NHF DAILY

NHF 70th Annual Bleeding Disorders Conference • Orlando, Florida • Highlights Edition

INSIDE

New Name, New Hopes for the Future

By Sandy Smith

At 70, NHF's annual event turns its eyes firmly to the future. And that starts with the new name: NHF's Bleeding Disorders Conference.

"This is the first time we've come together under our new name, a name that reflects everyone in our community, and signifies that NHF is an organization dedicated to all bleeding disorders," said NHF CEO Val D. Bias at the NHF 70th Bleeding Disorders Conference Opening Session on October 11.

Last year, 306 attendees identified as having a bleeding disorder other than hemophilia and von Willebrand disease. In recent years, education has been available for those with factor X and factor XIII, but this year adds a track for those with rare bleeding disorders, Bias said. "As more people become aware of the bleeding disorders community, more people with rare bleeding disorders will look to us for support."

As part of that expansion, Bias announced that all NHF walks will now be known as "Unite for Bleeding Disorders." He told the story of a father of two children with VWD questioning whether his family was "allowed" to go to the walk. "No member of our community should feel that way."

Throughout the session, Bias introduced guest speakers who

Continued on page 12

NHF CEO Val D. Bias looks on while the Joyful Noise Choir performs "Stand By Me" during the NHF 70th Annual Bleeding Disorders Conference Opening Session.

Chris Bombardier gets a high five from Graham Brayshaw, 5, after the first community screening of Bombardier Blood*, documenting Bombardier's inspirational journey as the first person with severe hemophilia to summit Mount Everest.*

Bombardier Blood: The View from a Higher Perspective

By Fiona Soltes

Truth be told, there are a number of moments in the documentary film *Bombardier Blood* in which Chris Bombardier fights back tears and struggles to regain composure along the way to reaching the Seven Summits, the highest peaks of the world's continents.

Early October 13, during the NHF 70th Annual Bleeding Disorders Conference, he found himself doing much the same.

Standing before a clearly moved and inspired crowd of supporters, friends, family and fans, Bombardier, who has severe hemophilia B, searched for words of thanks. He had just joined the group in watching the first community screening of the movie, which will be released to the general public in 2019. It is the emotional, poignant, nail-biting, raw, stirring and sometimes humorous tale of how he became the first person with severe hemophilia to summit Mount Everest in Nepal—much less all seven peaks. (Fewer than 500 people worldwide have accomplished the latter feat.) Octapharma sponsored the film, in addition to Bombardier's climb of Mount Everest and Mount Vinson in Antarctica; the film was presented as

Continued on page 13

NOW AVAILABLE

BAYER

Jivi®

antihemophilic factor
(recombinant) PEGylated-aucl

To learn more, visit www.jivi.com.

Starting the Mental Health Conversation

By Deb Burrows, BS, MA, EMTP

Debbie de la Riva, LPC, discusses Mental Health First Aid: How to Ask "Are You OK?"

Why did you pick this session?" Debbie de la Riva, LPC, asked the audience as she opened the session **Mental Health First Aid: How to Ask "Are You OK?"** Audience answers ranged from being a teacher and wanting to know what to look for and being a parent of a middle-schooler, to being a resident assistant at a university.

Here's what we know, continued de la Riva: The prevalence of depression in men with hemophilia shows that of 41 in a research study, 37 percent met the criteria for depression and 76 percent of those said it had significantly impacted their life.

The most common complication of chronic conditions in general is depression. "We don't talk about mental illness for a variety of reasons," de la Riva said. The reasons include: shame; feeling it is a character flaw; that one should be able to get over depression by a matter of will; it is not real because you cannot see it; and every organ can break down except for the brain.

One in five Americans experiences a mental health condition in any given year, de la Riva said. "Fifty-nine percent of those do not seek treatment. Of those who eventually get help, it takes an average of 10 years to do so." She added that most psychotic episodes happen in young adulthood, yet 48 percent of young adults with hemophilia have difficulty accessing their HTC. This may be because it is the time in many young adults' lives that they head off to college or move out on their own.

"We are doing great in the area of maintaining our physical health," she said. "But what about the cognitive, psychological, and behavioral aspects? How do you think about your disorder? How do you feel about it?"

The audience broke into groups of two and described their interpretation of "mental illness" to each other, before sharing back with the larger group: "Anything that affects ability to perceive, store, or recall information." "Not being able to trust your own mind."

De la Riva clarified the definition: "A mental disorder that is diagnosable and affects a person's thinking, emotional state, and behavior; disrupts the ability to carry out daily activities; the duration is significant, and symptoms are pervasive."

How can we prevent the feelings of isolation and deaths by suicide? De la Riva feels that the course, Mental Health First Aid, is one way. "This course needs to be as common as CPR courses."

Mental Health First Aid is a five-step action plan to reach out and have a compassionate and effective conversation with someone dealing with a mental health condition. This program helps one to recognize the signs and symptoms of someone living with a mental health condition or crisis, and then provides the resources for information and care.

According to de la Riva, this program has been in the U.S. since 2008. There are 12,000 mental health instructors. The five steps include:

- Assess for risk of suicide or harm
- Listen nonjudgmentally
- Give reassurance and information
- Encourage appropriate professional help
- Encourage self help and other support strategie

Attendees practiced steps with partners after de la Riva provided background "patient" information.

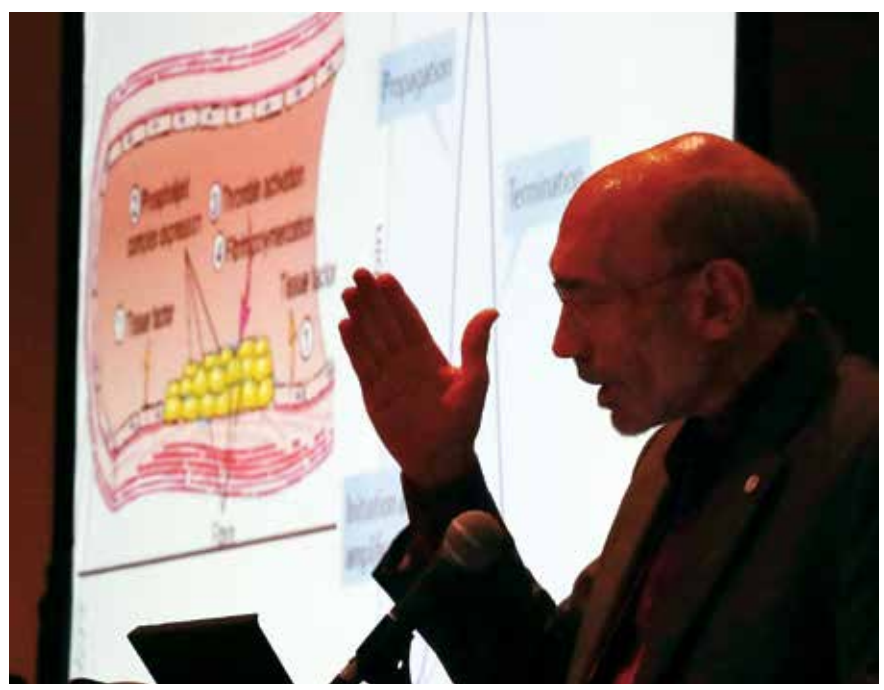
"The Mental Health First Aid program has given me a different perspective on how to approach difficult situations when working not just with community members in my role as programs and events manager for the New York City Hemophilia Chapter, but with people in general," said Marc Pangilinan. "Rather than immediately judging or dismissing someone for disruptive behavior, I learned how to both approach from an empathetic point of view and speak from a place of compassion and understanding to see what is causing that behavior. I was also given instructions on how to peacefully de-escalate the situation by guiding them towards the help they need."

Bob Robinson, executive director of Bleeding Disorders Alliance Illinois, added, "Mental health touches each of us at some point in our lives. It is such a secret killer in our society. The skills shared during this training can help us open conversations in the bleeding disorders community that has the potential to save lives."

MYTAKE

"I'm here to meet other people, specifically those with von Willebrand disease, and to come to things geared toward women, as advocacy for myself. It helps me add more to my toolkit....Changing the name of the event to the Bleeding Disorders Conference was really nice, because it's always about hemophilia. It doesn't hurt my feelings. I don't feel left out. But what it really means to me is that there's a dialogue around it....Some hematologists know more about hemophilia than VWD, and don't seem to be as comfortable treating it, or they have misconceptions about it. I'd like to see the community around VWD grow."

SOPHIA CIERI
NEW YORK



Glenn Pierce, MD, PhD, discusses exciting times in bleeding disorders research while presenting Advancements in Research: Now and the Future.

Finding Opportunities

By Deb Burrows, BS, MA, EMTP

Panel members from the National Youth Leadership Institute (NYLI) came together with teens, parents, and others interested in learning more about scholarship applications, resumes, and the NYLI program, on October 13 at the session **Free Rides and Better Jobs: Making the Most of What You've Got**.

Joelle Smith started the conversation by explaining the best places to find scholarship information. "There are so many areas to find scholarships information," Smith said. "Of course, one place is to go to your high school website. The NHF website also has a scholarship page under the community resources tab."

Matthew Delaney explained that when looking for scholarships, it is important to look at the description of the scholarship to see eligibility requirements. "Some will specify college types or fields of study, but most scholarships are there to help youth with bleeding disorders succeed. Make sure you meet the criteria to have the best chance of receiving a scholarship." He added that it helps to be as personal as possible, so you stand out among the applicants.

Having a good resume and showing that you are active in your school and your community is also a plus, according to Delaney. "A letter of recommendation might sound scary, but it is not." He suggested asking a guidance counselor, chapter director, or teacher to write the letter. "Go in a month or two in advance of when the letter is due to give the person plenty of time to write the letter. You may also need a letter from a physician." He suggested applying for as many scholarships as possible, and to send a photo of yourself being active at NHF Conference or chapter walk. Moderator Marlee Whetten added that all four years of her college was paid for through bleeding disorder scholarships.

Matthew Delaney (from left), Joelle Smith, Chelsee Nabritt, and Allison Albright discuss obtaining financial aid for college during Free Rides and Better Jobs.

Volunteer work is important to include on a resume, said Smith. "You can volunteer in your chapter to help with a walk or volunteer at camp. The people you meet through volunteer work can also write letters of recommendation."

Chelsee Nabritt and Allison Albright explained what goes into a good resume.

Whetten described the three-year NYLI program. Applications are open until October 31. "This is a leadership as well as a preprofessional program for youth (18-24 years old) in the bleeding disorders community," she said. "It is like a mini graduate program and the skills gained will set you up for success in your careers."

Medical Marijuana: Positive Impacts, Potent Questions

By Sandy Smith

Medical marijuana is legal in a majority of states – but that does not mean cannabis is widely available or understood.

"Our state and federal laws do not match and that's where we have barriers," said Michelle Witkop, DNP, FNP-BC, NHF's head of research, during the Pain Series session, **Medical Marijuana** on October 12. "As you cross over state lines, you have issues if you carry marijuana. If you fly across state lines, you're breaking a federal law."

The discrepancy between state and federal laws means researchers who rely on federal funding cannot study the efficacy of marijuana for treating pain.

But there is enough known about medical marijuana and its uses through the years. Witkop said prior to 1930, medical marijuana was legal, with "no constraints on it." In the early part of the last century, it became politicized." A 1937 tax, strongly opposed by the American Medical Association, made marijuana more difficult to get. Marijuana become even more politicized until 1970, when the Narcotics Act was passed, and marijuana made the list as a Schedule 1 drug.

Michelle Witkop, DNP, FNP-BC, (left) and Samantha Carlson, LMSW, present Pain Series: Medical Marijuana.

"Schedule 1 drugs have no medical use and a high potential for misuse," Witkop said. Marijuana is in the same category as drugs like heroin, LSD and ecstasy.

Still, organizations and commissions have seen uses for medical marijuana, though that has not yielded changes at the federal level, she said.

What is scientifically known about marijuana and its impact on the body is that it could prove helpful in a number of uses – without the same issues that other drugs have. Witkop compared the opioid receptor to the endocannabinoid system, which was only discovered in 1997. "This endocannabinoid system tries to keep your body in balance. It works on your immune system, your memory, your mood. Unlike the opioid receptors, the endocannabinoid system is throughout your body, but not in your body and lungs, so you don't have respiratory depression like you see with opioids."

Witkop likened the endocannabinoid system to a "cleaning system for your body." For those with bleeding disorders, the impact on pain can be profound. "It quiets down the cell. It's there when you need it. It's not something that you need to take more of."

Medical marijuana differs from recreational marijuana, particularly in the levels of CBD and THC. Medical marijuana tends to be higher in CBD, which has been shown to have a positive impact on pain, said Samantha Carlson, LMSW, director of patient support services at West Michigan Cancer Center & Institute for Blood Disorders.

In Michigan, where medical marijuana has been legalized, patients have been able to forgo narcotics in favor of the herb, Carlson said. She urged those seeking information on medical marijuana to engage their treatment team. Issues like how marijuana is be consumed – via edibles, vapes, topical or smoking – can vary its efficacy.

Given the varied laws – even, sometimes, at the local level – it is vital to understand what is allowed, Carlson said. She encouraged people to pursue information from credible sources. "When you're looking on the internet, make sure you're looking for medical marijuana education that is nonprofit. They're not trying to sell you anything."

The future use of medical marijuana is yet to be determined. All the same, Carlson believes that there is a "momentum in our country."

Your dreams. Our dedication.

At Shire we are driven to help improve the lives of members of the bleeding disorders community. You inspire us. Each pioneering new product and program represents another step toward our ultimate goal: **a life full of dreams and free of bleeds.**

Michelle
vWD
Nevada

Shire

bleedingdisorders.com

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated]

ADYNOVATE® is FDA approved for children and adults with Hemophilia A

PROVEN PROPHYLAXIS + SIMPLE,* TWICE-WEEKLY DOSING SCHEDULE = *moments* YOUR WAY

*ADYNOVATE allows you to infuse on the same 2 days every week. Work with your doctor to determine an infusion schedule that is appropriate for you.

The pediatric study of children <12 years of age (N=66) evaluated the immunogenicity, efficacy, PK (as compared to ADVATE® [Antihemophilic Factor (Recombinant)]), and safety of ADYNOVATE twice-weekly prophylaxis (40-60 IU/kg) and determined hemostatic efficacy in the treatment of bleeding episodes for 6 months.[1,2]

The pivotal trial of children and adults ≥12 years (N=137) evaluated the efficacy, PK, and safety of ADYNOVATE twice-weekly prophylaxis (40-50 IU/kg) vs on-demand (10-60 IU/kg) treatment, and determined hemostatic efficacy in the treatment of bleeding episodes for 6 months.[1]

+Children (<12 years) experienced a median overall ABR of 2.0 (IQR: 3.9) and a median ABR of zero for both joint (IQR: 1.9) and spontaneous (IQR: 1.9) bleeds[1,3]

+38% (n=25) of children (<12 years) experienced zero total bleeds; 73% (n=48) experienced zero joint bleeds; and 67% (n=44) experienced zero spontaneous bleeds[1]

Talk to your doctor to see if ADYNOVATE treatment may be right for you and visit ADYNOVATE.com

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated] Important Information

What is ADYNOVATE?

- ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency).
- Your healthcare provider (HCP) may give you ADYNOVATE when you have surgery.
- ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

Who should not use ADYNOVATE?

Do not use ADYNOVATE if you:

- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE [Antihemophilic Factor (Recombinant)]

Tell your HCP if you are pregnant or breastfeeding because ADYNOVATE may not be right for you.

What should I tell my HCP before using ADYNOVATE?

Tell your HCP if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADYNOVATE passes into your milk and if it can harm your baby.
- Are or become pregnant. It is not known if ADYNOVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

What important information do I need to know about ADYNOVATE?

- You can have an allergic reaction to ADYNOVATE. Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.
- Do not attempt to infuse yourself with ADYNOVATE unless you have been taught by your HCP or hemophilia center.

What else should I know about ADYNOVATE and Hemophilia A?

- Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE from working properly. Talk with your HCP to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

What are possible side effects of ADYNOVATE?

- The common side effects of ADYNOVATE are headache and nausea. These are not all the possible side effects with ADYNOVATE. Tell your HCP about any side effects that bother you or do not go away.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see Important Facts about ADYNOVATE on the following page and discuss with your HCP.

For full Prescribing Information, visit www.ADYNOVATE.com.

References: 1. ADYNOVATE Prescribing Information. **2.** Mullins ES, Stasyshyn O, Alvarez-Román MT, et al. Extended half-life pegylated, full-length recombinant factor VIII for prophylaxis in children with severe haemophilia A. *Haemophilia*. 2016 Nov 27. doi: 10.1111/hae.13119 [Epub ahead of print]. **3.** Data on file.

ADYNOVATE
[Antihemophilic Factor (Recombinant), PEGylated]

Patient Important facts about

ADYNOVATE® [Antihemophilic Factor (Recombinant), PEGylated]

This leaflet summarizes important information about ADYNOVATE. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider, and it does not include all of the important information about ADYNOVATE. If you have any questions after reading this, ask your healthcare provider.

What is the most important information I need to know about ADYNOVATE?

Do not attempt to do an infusion to yourself unless you have been taught how by your healthcare provider or hemophilia center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing ADYNOVATE so that your treatment will work best for you.

What is ADYNOVATE?

ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ADYNOVATE when you have surgery. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

Who should not use ADYNOVATE?

You should not use ADYNOVATE if you:

- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE® [Antihemophilic Factor (Recombinant)]

Tell your healthcare provider if you are pregnant or breastfeeding because ADYNOVATE may not be right for you.

How should I use ADYNOVATE?

ADYNOVATE is given directly into the bloodstream.

You may infuse ADYNOVATE at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia A learn to infuse their ADYNOVATE by themselves or with the help of a family member.

Your healthcare provider will tell you how much ADYNOVATE to use based on your individual weight, level of physical activity, the severity of your hemophilia A, and where you are bleeding.

Reconstituted product (after mixing dry product with wet diluent) must be used within 3 hours and cannot be stored or refrigerated. Discard any ADYNOVATE left in the vial at the end of your infusion as directed by your healthcare professional.

You may have to have blood tests done after getting ADYNOVATE to be sure that your blood level of factor VIII is high enough to clot your blood.

How should I use ADYNOVATE? (cont'd)

Call your healthcare provider right away if your bleeding does not stop after taking ADYNOVATE.

What should I tell my healthcare provider before I use ADYNOVATE?

You should tell your healthcare provider if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADYNOVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADYNOVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

What are the possible side effects of ADYNOVATE?

You can have an allergic reaction to ADYNOVATE.

Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

The common side effects of ADYNOVATE are headache and nausea. Tell your healthcare provider about any side effects that bother you or do not go away.

These are not all the possible side effects with ADYNOVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADYNOVATE and Hemophilia A?

Your body may form inhibitors to Factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to Factor VIII.

Medicines are sometimes prescribed for purposes other than those listed here. Do not use ADYNOVATE for a condition for which it is not prescribed. Do not share ADYNOVATE with other people, even if they have the same symptoms that you have.

The risk information provided here is not comprehensive. To learn more, talk with your health care provider or pharmacist about ADYNOVATE. The FDA-approved product labeling can be found at www.shirecontent.com/PI/PDFs/ADYNOVATE_USA_ENG.pdf or 855-4-ADYNOVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Baxalta US Inc.
Westlake Village, CA 91362 USA
U.S. License No. 2020
Issued 12/2016

16I045-ADY-US S39276 04/18

Shire

Update on Washington Policies

By Deb Burrows, BS, MA, EMTP

Johanna Gray, MPA, federal policy advisor to NHF, presented an update on reforms and potential changes to healthcare policies at the **Washington Insider** session on October 12.

To begin, Gray provided an overview of some of the many federal agencies that affect the bleeding disorders community, including the Food and Drug Administration (FDA); National Heart, Lung, and Blood Institute (NHLBI); the Centers for Disease Control and Prevention (CDC); and the Health Resources and Services Administration (HRSA). These agencies, working separately and together, have programs that affect the bleeding disorders community, including research, education, outreach, blood safety, and drug approval.

The budgets for these agencies are determined by Congress. "Congress passed a full year health funding bill before October 1 for fiscal year 2019 for the first time in 22 years," announced Gray. "This allows our HTCs and researchers to have predictable funding."

AFFORDABLE CARE ACT (ACA)

Gray provided an update on the Affordable Care Act. While Congress considered several bills that would have repealed the ACA in 2017, none passed. Gray conveyed that in December 2017, Congress passed a tax reform bill that zeroed out the individual mandate penalty for not having insurance, which will go into effect in 2019. As a result of this change, a new lawsuit, Texas vs. Azar, has been filed that seeks to declare the ACA unconstitutional. In addition, the Trump administration has released new rules to expand access to association health plans and short-term limited duration insurance plans. These plans are not required to maintain ACA patient protections, such as lifetime caps and pre-existing conditions.

> "Check to see if prescription drugs are covered, check your access to providers, and check what limits to service may exist."

She cautioned that these types of plans offer less coverage, even though they are cheaper. "I encourage everyone to read the fine print." She suggested looking at the details and cautioned not to buy a plan simply because it costs less. "Check to see if prescription drugs are covered, check your access to providers, and check what limits to service may exist."

MEDICAID

NHF's public policy team is working diligently to address restrictive Medicaid policies. "States can apply for Section 1115 Waivers to tailor their Medicaid programs to their populations and the Trump administration is willing to approve these." Eligibility and enrollment restrictions have been approved in some states and are pending in others. Work requirements for Medicaid coverage are also a concern for the bleeding disorders community. "Talk to your local chapters or NHF's public policy team for more information about what is happening in your state," Gray said.

Speaker Johanna Gray, MPA, and moderator Nathan Schaefer present the latest healthcare policy issues in Washington, DC, during Washington Insider.

340B REFORM

The 340B Drug Discount Program was established in 1992. As HRSA grantees, HTCs were designated as covered entities eligible to participate in this program, which allows them to purchase discounted drugs and generate program income to support the comprehensive care offered at their centers. "Due to growth in 340B participation since 2010, there has be growing scrutiny in Washington and thus there is a potential for reforms to 340B."

Gray told attendees that this increased scrutiny of the 340B program is not targeted at HTCs. However, she warned that there was potential for unintended consequences of 340B reform legislation.

"NHF will continue to monitor these issues and provide updates. If you have any questions or concerns about coverage, email NHF at advocate@hemophilia.org," concluded Gray.

Gray ended with a reminder that the midterm elections would bring changes to Washington. She echoed the sentiment from NHF CEO Val D. Bias at Thursday's Opening Session, encouraging attendees to both vote and get involved in advocating for their rights as patients.

Want to get involved? NHF's Washington Days is March 27 – 29, 2019.

Researchers Advance Science through *My Life, Our Future* Research Repository

This year, we were lucky to have some of the first researchers that were granted access to the *My Life, Our Future (MLOF)* Research Repository on-site with us in Orlando. The resource, the largest of its kind in the world, contains data and blood samples from more than 9,000 people affected by hemophilia and is now being utilized to advance scientific understanding of hemophilia.

During the dedicated *MLOF* panel session, **MLOF: Advancing Science**, NHF CEO Val E. Bias thanked participants for their critical role in the program. He also highlighted the value of research programs like *MLOF* in furthering progress for those impacted by bleeding disorders, noting, "The great thing about this community is, if we wish it, if we desire it, if we have passion for it, it does come true. And that's why one day, we will have a cure."

Following his remarks and an overview of *MLOF*'s successes to date, several researchers provided an overview of their studies:

Samuel Lessard, PhD, of Bioverativ who will be exploring genes associated with inhibitor development in people with hemophilia A

Hong Yang, PhD, of the FDA, whose work will focus on predicting inhibitor development through mathematical modeling

Jill Johnsen, MD, of Bloodworks Northwest Research Institute, who will research factors associated with bleeding in female hemophilia carriers

The gathering was moderated by Moses Miles of the American Thrombosis and Hemostasis Network and ended with an interactive Q&A session.

For more information about *MLOF* and ongoing updates about these and other approved research projects utilizing the *MLOF* Research Repository, visit www.MyLifeOurFuture.org.

Austin Baptiste hones his infusion technique with a practice arm along with his parents Michael and Heather Baptiste during Infusion Game Plan.

Brian and Carlisa Magee attend the Basics of FX Deficiency session with their daughter Ariel.

Sue Martin saves her speech for posterity in the NHF time capsule, which will be opened in 30 years during the 100th NHF Bleeding Disorders Conference.

Logan Bularz (left) and Riley Blair participate in an interactive activity at Teen Track Kickoff.

Trinity Edgemon takes the wheel to steer the Magical Train during the Exhibit Hall Welcome Reception.

NHF attendees watch the first community screening of Bombardier Blood, *documenting Chris Bombardier's inspirational journey as the first person with severe hemophilia to summit Mount Everest.*

UNLOCKING YOUR SELF-POTENTIAL

ONLY ADVATE® HAS 15 YEARS OF EXPERIENCE IN THE REAL WORLD AS A RECOMBINANT FACTOR VIII[1]

- Proven in a pivotal clinical trial to reduce the number of bleeding episodes in children and adults when used prophylactically[2]*
- Third-generation full-length molecule, similar to the factor VIII that occurs naturally in the body[1,2]

*Multicenter, open-label, prospective, randomized, 2-arm controlled trial of 53 previously treated patients with severe to moderately severe hemophilia A. Two different ADVATE prophylaxis regimens (standard, 20-40 IU/kg every 48 hours, or pharmacokinetic-driven, 20-80 IU/kg every 72 hours) were compared with on-demand treatment. Patients underwent 6 months of on-demand treatment before 12 months of prophylaxis.[2]

The market leader in hemophilia A treatment
(Based on 2016 data published July 2017)[3]

Learn more at ADVATE.com

ADVATE Important Information

What is ADVATE?

- ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called "classic" hemophilia).
- ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A. Your healthcare provider (HCP) may give you ADVATE when you have surgery.
- ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

Who should not use ADVATE?

Do not use ADVATE if you:

- Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your HCP if you are pregnant or breastfeeding because ADVATE may not be right for you.

What should I tell my HCP before using ADVATE?

Tell your HCP if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADVATE passes into your milk and if it can harm your baby.
- Are or become pregnant. It is not known if ADVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

What important information do I need to know about ADVATE?

- You can have an allergic reaction to ADVATE. Call your HCP right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.
- Do not attempt to infuse yourself with ADVATE unless you have been taught by your HCP or hemophilia center.

What else should I know about ADVATE and Hemophilia A?

- Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Talk with your HCP to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

What are possible side effects of ADVATE?

- Side effects that have been reported with ADVATE include: cough, headache, joint swelling/aching, sore throat, fever, itching, unusual taste, dizziness, hematoma, abdominal pain, hot flashes, swelling of legs, diarrhea, chills, runny nose/congestion, nausea/vomiting, sweating, and rash. Tell your HCP about any side effects that bother you or do not go away or if your bleeding does not stop after taking ADVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA.
Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

For additional safety information, please see Important Facts about ADVATE on the following page and discuss with your HCP.

For Full Prescribing Information, visit www.ADVATE.com.

References: 1. Grillberger L, Kreil TR, Nasr S, Reiter M. Emerging trends in plasma-free manufacturing of recombinant protein therapeutics expressed in mammalian cells. *Biotechnol J.* 2009;4(2):186-201. **2.** ADVATE Prescribing Information. **3.** The Marketing Research Bureau, Inc. The plasma proteins market in the United States. 2016.

Shire

ADVATE
[Antihemophilic Factor (Recombinant)]

ADVATE
[Antihemophilic Factor (Recombinant)]

Important facts about

ADVATE [Antihemophilic Factor (Recombinant)]

This leaflet summarizes important information about ADVATE. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider, and it does not include all of the important information about ADVATE. If you have any questions after reading this, ask your healthcare provider.

What is the most important information I need to know about ADVATE?

Do not attempt to do an infusion to yourself unless you have been taught how by your healthcare provider or hemophilia center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing ADVATE so that your treatment will work best for you.

What is ADVATE?

ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called "classic" hemophilia). The product does not contain plasma or albumin. Hemophilia A is an inherited bleeding disorder that prevents blood from clotting normally.

ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A.

Your healthcare provider may give you ADVATE when you have surgery. ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

Who should not use ADVATE?

You should not use ADVATE if you:

- Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your healthcare provider if you are pregnant or breastfeeding because ADVATE may not be right for you.

How should I use ADVATE?

ADVATE is given directly into the bloodstream.

You may infuse ADVATE at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia A learn to infuse their ADVATE by themselves or with the help of a family member.

Your healthcare provider will tell you how much ADVATE to use based on your weight, the severity of your hemophilia A, and where you are bleeding.

You may have to have blood tests done after getting ADVATE to be sure that your blood level of factor VIII is high enough to clot your blood.

Call your healthcare provider right away if your bleeding does not stop after taking ADVATE.

What should I tell my healthcare provider before I use ADVATE?

You should tell your healthcare provider if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

What are the possible side effects of ADVATE?

You can have an allergic reaction to ADVATE.

Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

Side effects that have been reported with ADVATE include:

cough	headache	joint swelling/aching
sore throat	fever	itching
unusual taste	dizziness	hematoma
abdominal pain	hot flashes	swelling of legs
diarrhea	chills	runny nose/congestion
nausea/vomiting	sweating	rash

Tell your healthcare provider about any side effects that bother you or do not go away.

These are not all the possible side effects with ADVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADVATE and Hemophilia A?

Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

Medicines are sometimes prescribed for purposes other than those listed here. Do not use ADVATE for a condition for which it is not prescribed. Do not share ADVATE with other people, even if they have the same symptoms that you have.

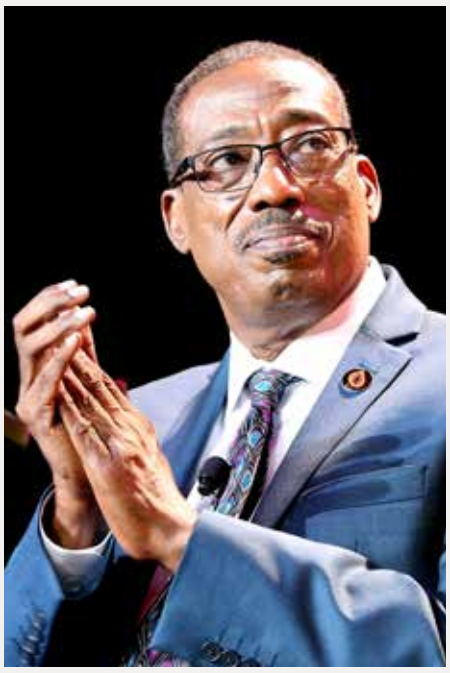
The risk information provided here is not comprehensive. To learn more, talk with your health care provider or pharmacist about ADVATE. The FDA approved product labeling can be found at www.ADVATE.com or 1-888-4-ADVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Baxalta US Inc.
Westlake Village, CA 91362 USA
U.S. License No. 2020
Issued: 11/2016

S42038 08/18

Shire



NHF CEO Val D. Bias applauds during the Opening Session.

Michelle Kim and Ryan White's mother, Jeanne White-Ginder, at the Bleeding Disorders Conference Opening Session.

Brian Andrews, chair of the board, speaks at the Opening Session.

New Name, New Hopes

Continued from page 1

demonstrated the diversity of those impacted by bleeding disorders. "It is my hope that by hearing from them, you'll be struck, as I was, by the range of different voices that make up this community and the great potential that each voice contains."

Everyone at the conference can make their voices heard, too. NHF board chair Brian Andrew announced a time capsule, which will capture the community's hopes for the future. It will be opened at NHF's 100th anniversary. Bias said he probably will open the capsule before then, logging each participant's hopes "just to remind me of our path."

> **The bleeding disorders community is growing and changing, and we must grow and change with it.**

RESEARCH AGENDA

Bias announced several initiatives and moves that will keep NHF focused on the broader bleeding disorders community.

Research will focus on partnerships, using the *My Life, Our Future* genotyping campaign as a template that will expand beyond hemophilia. "NHF's mission is to represent your voice. As we move forward with our research agenda, we are offering you a seat at the table. I invite you to come in, sit down, and be a part of the conversation."

That conversation will be global. "We realized that we could do more to help people with bleeding disorders around the world," Bias said. "And it would be selfish to think otherwise."

NHF is participating in the World Federation of Hemophilia's Youth Group Twinning Pilot Project, working with Hemophilia Federation India to create a youth development program modeled on NHF's National Youth Leadership Institute.

"*My Life, Our Future* set out to genotype 5,000 people with hemophilia; the response yielded twice as many samples in the repository, which is now available for researchers.

At last year's event, Bias announced plans to double NHF's investment in research. He announced the two recipients of NHF's Innovative Investigator Awards, Shannon Meeks, MD, and Jill Johnsen, MD.

Meeks is working on delivery capsules that protect factor VIII from inhibitors as it travels to the site of a bleed before dissolving. Johnson is developing a handheld device that measures factor activity in real time, much the same as those with diabetes can measure blood sugar levels.

Bias also announced the establishment of Bridge Grants, supporting investigators whose work was denied National Institutes of Health funding due to budget constraints. "We will not let discovery in hematology fall behind due to lack of funding," Bias said.

Change also is occurring through NHF's work in conjunction the American Society of Hematology, the International Society on Thrombosis and Hemostasis, and the World Federation of Hemophilia to create guidelines for von Willebrand disease. "In many countries, there are no guidelines on how to test and diagnose VWD," Bias said. "If there is no way to diagnose it, then there is no way to treat it."

FUTURE FOCUSED

Steven Pipe, MD, chair of NHF's Medical and Scientific Advisory Council and medical director of the pediatric hemophilia and coagulation disorders program at the University of Michigan, detailed his hopes that people with bleeding disorders are on the cusp of true innovations in treatments.

"Dr. Pipe has tapped into the excitement many of us feel about this age of discovery that we are in now," Bias said. "With some truly extraordinary therapies available now or on the horizon, it can feel like we made a huge, sudden leap forward. These products aren't a sudden leap. But behind every breakthrough is the work of hundreds of scientists who pushed our understanding forward in increments. If each scientific breakthrough holds the work of hundreds of researchers, it also holds the hope of the people who need it."

MYTAKE

"I consider myself a part of new generation of advocates in our community. I think it is my responsibility to advocate for better diagnoses and treatments....My hope is that today we can start advocating and defend our right to live 'healthy' with our disease."

DANIELA DELGADO
Age 11

"I believe collaborating with NHF will uplift our youth leaders, strengthen their capabilities to lead, and most importantly, change the hemophilia landscape of India. Treatment for hemophilia in a developing country such as India is far from ideal....I am hopeful that this seed of learning being planted by NHF in my organization will bear much fruit in the future."

MUKESH GARODIA
Vice President of Hemophilia Federation India

Getting Gender-Specific With 'Women and Our Bodies'

By Fiona Soltes

The session **Women and Our Bodies** on October 11 started with the very basics: a relatively lighthearted review of female anatomy, complete with pictures and diagrams, as well as an overview of the menstrual cycle.

"It's amazing how many people come into the clinic and don't know what their parts are," said Judith Simms-Cendan, MD, professor of obstetrics and gynecology, University of Central Florida College of Medicine, Winner Palmer/Arnold Palmer Hospital.

Amazing, too, how many different versions of "normal" there are when it comes to periods – especially when a bleeding disorder is involved.

Shveta Gupta, MD, Arnold Palmer Hospital for Children, picked things up from there, offering a simple view of how clotting happens in the body, and what happens when there's a bleeding disorder. Common symptoms, she said, include heavy menstrual bleeding, nosebleeds, oral bleeding and bruising.

These symptoms are subjective, but the doctors agreed: "Whatever you say is too much," Gupta said, "is too much."

The good news is that there are options for controlling bleeding, and there are a variety of reasons for doing so.

Shveta Gupta, MD, gives her presentation at Women and Our Bodies.

"There's so much out there now available that wasn't in the past," Simms-Cendan said. "It's not all about pads and tampons."

She spoke about THINX period panties, for example, as well as menstrual cups which can be used for 12 hours without the risk of toxic shock or contributing to the landfill.

Birth control pills, progesterone IUDs, patches and rings also can help control bleeding, as well as manage other symptoms. There are therapies and factors, too, for those with heavy periods and bleeding disorders.

Bleeding disorders, however, are lifelong; the session also included insight on pre-conception, pregnancy and postpartum. Shaveta Malik, MD, University of Buffalo department of obstetrics and gynecology, talked about risks unique to those with bleeding disorders in these areas, and stressed conversations with hematologists and gynecologists from the start.

When a patient with a severe bleeding disorder wants to have a baby, then, the ideal situation is to minimize the time from the end of the birth control to the pregnancy.

Women also should know their carrier status, as well as the methods of diagnosing a potentially affected infant prior to delivery, Malik said.

Malik finished her portion of the presentation with a few suggested resources: the Foundation for Women and Girls with Bleeding Disorders (www.fwgbd.org); Bedsider (www.bedsider.org), a nonprofit which helps women find the right birth control methods for their lives and bodies; and the HFA Blood Sisterhood Mobile App (www.sisterhoodapp.com), a menstrual cycle tracker specifically for women with bleeding disorders.

After a brief break, Charletta A. Ayers, MD, MPH, department of obstetrics, gynecology and reproductive sciences, Rutgers Robert Wood Johnson Medical School, dove into the session's second half. It continued the age spectrum through childbearing to the golden years.

When women of childbearing age have bleeding disorders, Ayers said, it's important for doctors to not just dismiss any abnormalities as related to the bleeding disorder. She, too, is a proponent of IUDs for controlling bleeding.

Challenges can multiply as women with bleeding disorders go through menopause. Even after periods stop, those with bleeding disorders still should stay in touch with their doctors, Ayers said.

She summarized by saying, "You are the director of your health." At the same time, collaboration and communication with experts like hematologists and gynecologists is essential."

Everest

Continued from page 1

an Industry Symposium session.

From the opening scenes of a shaky self-infusion at the Mount Everest base camp—Bombardier still doesn't care much for needles—the film moves through the candid fears and concerns of family members and healthcare partners; the meetings with "blood brothers" in Nepal whose lives are significantly different without the same access to factor and services; the mental and physical preparations for the adventure; and the history of a boy simply looking for meaning and purpose when his dreams of playing baseball were stunted by his condition.

"I always wanted to do something different with my life," he admits in the film. He seemed as surprised as anyone else that this could be it.

"It's pretty emotional to watch this again, because all of those feelings of being up there come back to me," he told the crowd after the screening. "All of those feelings of representing the hemophilia community, especially those that don't have access to care, and of bringing awareness to that, which is really important to me."

> **"The film is nothing if it's not us being with Chris, as a community, as he climbs, carrying the history of this community, carrying the global community, carrying a lot."**

All told, the adventure raised $100,000 for the international nonprofit Save One Life, which provides financial assistance to children and youth with bleeding disorders in developing nations, and resulted in 100 kids being sponsored. And now, it reaches its next mountain: getting people to see it, so the story, the inspiration, the support and the increased understanding can continue.

Director Patrick James Lynch, who also has severe hemophilia, urged those in attendance to visit the film's website, www.bombardierblood.com; to host area screenings of the film; and to rent, purchase and share it with others when it becomes widely available. In addition, there's a raffle with a grand prize that includes a trip to Denver for the movie's Colorado premiere in December. The drawing will be Oct. 27, and tickets are available through www.saveonelife.net.

"It was important to me that this film captured what it meant for Chris to be climbing," said Lynch, CEO and cofounder of digital content agency Believe Limited. "The film is nothing if it's not us being with Chris, as a community, as he climbs, carrying the history of this community, carrying the global community, carrying a lot. And I think all of us who have hemophilia, and those of us who live in households with hemophilia, know how comprehensively it impacts your life. Not just physically, but mentally, emotionally, the trauma that you have to process and go through and learn to live with." His first "ask," he said, was for those who could support Save One Life to do so. "I don't think I need to tell you why," he said. Second, he hoped to see the film shared at chapter meetings and other gatherings. "We then have a chance for a domino effect, and to put this film, and hemophilia, and our community's story, in front of as many people as earthly possible. But I can't do that. I need the community to help me do that."

FEIBA [Anti-Inhibitor Coagulant Complex] Indications and Detailed Important Risk Information for Patients

Indications for FEIBA

FEIBA is an Anti-Inhibitor Coagulant Complex approved for use in hemophilia A and B patients with inhibitors for:

- Control and prevention of bleeding episodes
- Use around the time of surgery
- Routine prophylaxis to prevent or reduce the frequency of bleeding episodes.

FEIBA is NOT for use in the treatment of bleeding episodes resulting from coagulation factor deficiencies without inhibitors to factor VIII or factor IX.

Detailed Important Risk Information for FEIBA

WARNING: EVENTS INVOLVING CLOTS THAT BLOCK BLOOD VESSELS

- **Blood clots that block blood vessels and their effects have been reported during post-marketing surveillance following infusion of FEIBA, particularly following administration of high doses (above 200 units per kg per day) and/or in patients at risk for forming blood clots.**
- **If you experience any of these side effects, call your doctor right away.**

Who should not use FEIBA?

You should not use FEIBA if:

- You had a previous severe allergic reaction to the product
- You have Disseminated Intravascular Coagulation (DIC), or signs of small blood vessel clots throughout the body
- You have sudden blood vessel clots or blocked blood vessels, (such as, heart attack or stroke)

What other important information should I know about FEIBA?

Events involving blood clots blocking blood vessels (such as blood clot in vein, blood clot in the lung, heart attack, and stroke) can occur with FEIBA, particularly after receiving high doses (above 200 units per kg per day) and/or in patients with risk factors for clotting.

Events of thrombotic microangiopathy (TMA), a condition where blood clots and damage occur in small blood vessels, were reported in an emicizumab (Hemlibra®) clinical trial where patients received FEIBA with emicizumab as part of a treatment plan for breakthrough bleeding. The safety and efficacy of FEIBA for breakthrough bleeding in patients receiving emicizumab has not been established. If you take, or anticipate taking, FEIBA with emicizumab, tell your doctor, since they will need to closely monitor you.

At first sign or symptom of a sudden blood vessel clot or blocked blood vessel (such as chest pain or pressure, shortness of breath, fever, altered consciousness, vision, or speech, limb or abdomen swelling and/or pain), stop FEIBA administration right away and seek immediate emergency medical treatment.

Infusion of FEIBA should not exceed a single dose of 100 units per kg body weight and daily doses of 200 units per kg of body weight. Maximum injection or infusion rate must not exceed 2 units per kg of body weight per minute.

Allergic reactions, including severe, sometimes fatal allergic reactions that can involve the whole body, can occur following the infusion of FEIBA. Stop using FEIBA promptly and call your doctor or get emergency treatment right away if you get a rash, hives or welts, experience itching, tightness of the throat, vomiting, abdominal pain, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

Because FEIBA is made from human plasma it may carry a risk of transmitting infectious agents, such as viruses, variant Creutzfeldt-Jakob disease (vCJD) and, theoretically, the Creutzfeldt-Jakob disease (CJD).

What are the possible side effects of FEIBA?

The most common side effects observed during the prophylaxis clinical study were low number of red blood cells, diarrhea, bleeding into a joint, positive test for hepatitis B surface antibodies, nausea, and vomiting.

The serious side effects seen with FEIBA are allergic reactions and clotting events involving blockage of blood vessels, which include stroke, blockage of the main blood vessel to the lung, and deep vein blood clots.

Call your doctor right away about any side effects that bother you during or after you stop taking FEIBA.

What other medications might interact with FEIBA?

Talk with your doctor about the possibility of formation of blood clots when taking drugs that may prevent clot breakdown such as tranexamic acid, and aminocaproic acid. There have not been adequate studies of the use of FEIBA and rFVIIa (NovoSeven®), or emicizumab together, or one after the other. Use of drugs that may prevent clot breakdown within approximately 6 to 12 hours after the administration of FEIBA is not recommended.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see next page for Important Facts about FEIBA.

References: 1. Pergantou H, Matsinos G, Papadopoulos A, Platokouki H, Aronis S. Comparative study of validity of clinical, X-ray and magnetic resonance imaging scores in evaluation and management of haemophilic arthropathy in children. Haemophilia. May 2006;12(3):241-247. **2.** Gringeri A, Ewenstein B, Reininger A. The burden of bleeding in haemophilia: is one bleed too many? Haemophilia. Jul 2014;20(4):459-463. **3.** FEIBA Prescribing Information. **4.** Antunes SV, Tangada S, Stasyshyn O, et al. Randomized comparison of prophylaxis and on-demand regimens with FEIBA NF in the treatment of haemophilia A and B with inhibitors. Haemophilia. 2014;20(1):65-72.

Shire

Important Facts about FEIBA® (Anti-Inhibitor Coagulant Complex)

What is FEIBA used for?

FEIBA (Anti-Inhibitor Coagulant Complex) is used for people with Hemophilia A and B with Inhibitors to control and prevent bleeding episodes, around surgery, or routinely to prevent or reduce the number of bleeding episodes. It is NOT used to treat bleeding conditions without inhibitors to Factor VIII or Factor IX.

When should I not take FEIBA?

You should not take FEIBA if you have had hypersensitivity or an allergic reaction to FEIBA or any of its components, including factors of the kinin generating system, if you have a condition called Disseminated Intravascular Coagulation (DIC), which is small blood clots in various organs throughout the body, or currently have blood clots or are having a heart attack. Make sure to talk to your healthcare provider about your medical history.

What Warnings should I know about FEIBA?

FEIBA can cause blood clots, including clots in the lungs, heart attack, or stroke, particularly after high doses of FEIBA (above 200 units per kg per day) or in people with a high risk of blood clots. Patients that have a risk of developing blood clots should discuss the risks and benefits of FEIBA with their healthcare provider since FEIBA may cause blood clots. Events of thrombotic microangiopathy (TMA), a condition where blood clots and damage occur in small blood vessels, were reported in an emicizumab (Hemlibra®) clinical trial where patients received FEIBA with emicizumab as part of a treatment regimen for breakthrough bleeding. The safety and efficacy of FEIBA for breakthrough bleeding in patients receiving emicizumab has not been established. If you take, or anticipate taking, FEIBA with emicizumab, tell your doctor, since they will need to closely monitor you. At first sign or symptom of a sudden blood vessel clot or blocked blood vessel (such as chest pain or pressure, shortness of breath, fever, altered consciousness, vision, or speech, limb or abdomen swelling and/or pain), stop FEIBA administration right away and seek immediate emergency medical treatment.

Allergic reactions, including severe, sometimes fatal allergic reactions that can involve the whole body, can occur following the infusion of FEIBA. Stop using FEIBA promptly and call your doctor or get emergency treatment right away if you get a rash, hives or welts, experience itching, tightness of the throat, vomiting, abdominal pain, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

Because FEIBA is made from human plasma, it may carry the risk of transmitting infectious agents, for example, viruses, including Creutzfeldt-Jakob disease (CJD) agent, and the variant CJD agent. Although steps have been taken to minimize the risk of virus transmission, there is still a potential risk of virus transmission.

What should I tell my healthcare provider?

Make sure to discuss all health conditions and medications with your healthcare provider. If you are pregnant or are planning to become pregnant, or are a nursing mother, make sure to talk with your healthcare provider for advice on using FEIBA.

What are the side effects of FEIBA?

The most frequent side effects of FEIBA are: low red blood cell count, diarrhea, bleeding into a joint, hepatitis B surface antibody positivity, nausea, and vomiting. The most serious side effects of FEIBA include: hypersensitivity reactions, including anaphylaxis, stroke, blood clots in the lungs, and blood clots in the veins. Always immediately talk with your healthcare provider if you think you are experiencing a side effect.

What other medications might interact with FEIBA?

Talk with your doctor about the possibility of formation of blood clots when taking drugs that may prevent clot breakdown such as tranexamic acid, and aminocaproic acid. There have not been adequate studies of the use of FEIBA and rFVIIa (NovoSeven®), or emicizumab together, or one after the other. Use of drugs that may prevent clot breakdown within approximately 6 to 12 hours after the administration of FEIBA is not recommended. For additional information on potential drug interaction with emicizumab, see the “What Warnings Should I Know about FEIBA?” section above.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088

The risk information provided here is not comprehensive. To learn more, talk about FEIBA with your healthcare provider or pharmacist. The FDA-approved product labeling can be found at

http://www.feiba.com/us/forms/feiba_pi.pdf or by calling 1-800-423-2090 and selecting option 5.

S43033 09/18

Avery Hoffman, 9, shows off her smile and painted face at the Final Night Event.

Bentley Shawen picks up a heart of love to add to the inside of his "furry friend" stuffed animal he made at the Final Night Event.

Aida Faraj (from left), Hadi Rihan, 10, Sarah Rihan, 6, and Imad Rihan pose for a family picture in the photo booth during the Final Night Event.

Laura Silva gives a "thumbs up" while taking in the Final Night Event.

The audience reacts to the Blues Brothers during the NHF's Official Bleeding Disorders Conference Awards Ceremony.

The Blues Brothers perform during the NHF's Official Bleeding Disorders Conference Awards Ceremony.

Lessons Learned: Navigating Through Schools

By Fiona Soltes

Interactive class was in session during the NHF 70th Annual Bleeding Disorders Conference as a nurse, a school board member whose son has hemophilia, and a teacher with numerous instances of von Willebrand disease in her immediate family talked about the realities of ensuring appropriate care throughout a child's education.

Navigating Through Schools covered all ages, from preschool through college and the workplace, stressing the importance of communication, protocols, partnerships and resources. Don't assume that anyone knows anything about bleeding disorders, the panelists said – and don't assume that something that works in one community or at one school will necessarily be the same in another.

Charmaine Biega, RN, hemophilia nurse coordinator, Nationwide Children's Hospital, spoke about visiting schools and meeting with physical education teachers, principals, school nurses and even bus drivers to share information. That goes beyond just sharing what bleeding disorders are, Biega said, to discussing what to look for, as well as basic first aid and treatments.

"You want to give them as much information as you can," she said. "But you don't want to scare them, either."

Handouts can be helpful; Marissa Levy, whose elementary-aged son has hemophilia, recommends NHF's "Playing It Safe – Bleeding Disorders, Sports and Exercise," for example.

Levy said she has learned how important it is to ensure her child's teachers have "a comfort level with blood." Her son, she said, had a teacher who had trouble even hearing the word "blood," which made discussing his care difficult. When he started school, HTC representatives came to talk to the principal, teacher and others.

After school begins, Levy advised, parents must ensure the school nurse has clear direction on who to call – and in what order – in case of any issues; school representatives also should know about protocol for minor versus major injuries. Joining the school board, she said, has allowed to advocate not only for her son, but also for others. It also has allowed her to monitor and address challenges as they arise.

Jessica Amende, meanwhile, is a Montana-based sixth-grade teacher who has VWD; her husband and two of her three children also have VWD. She spoke about the differences between a 504 plan and an Individualized Education Plan (IEP) for children with bleeding disorders. Both ensure that the child's needs are being met, both require ongoing dialogue, and both can extend through college. An IEP is for kids who qualify for special education services, whether due to learning disability, developmental delay, or other challenge. In general, the IEP requires a formal evaluation process and team meeting to establish. The 504 plan, on the other hand, is a bit more fluid; Amende and her husband have written those plans themselves, along with the school nurse.

Jessica Amende discusses issues involved with raising a child with bleeding disorder through the school years during Navigating Through Schools.

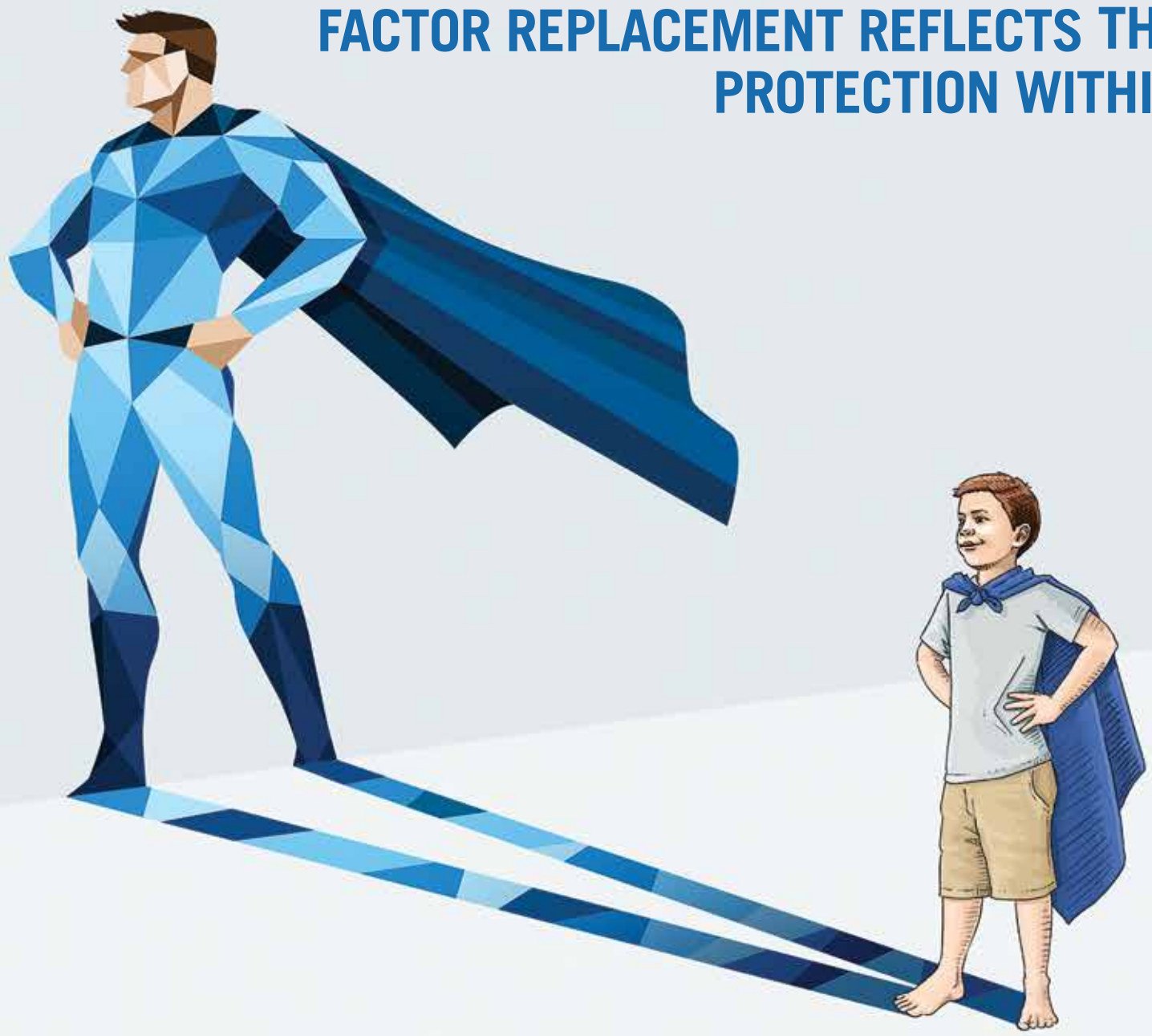
Regardless of what kind of plan is in place, however, it's important to remember that your child has rights. Even when educators mean well, they can make things worse by, say, excluding children from opportunities due to fear or misunderstanding.

"You have to become the expert," said Cathy Tiggs, MSSA, LISW, UH Cleveland Medical Center, who moderated the session. Ideally, the system "works." "But sometimes it doesn't work well at all, and you have to be your best advocate," Tiggs said. HTCs, social workers, providers and NHF are willing and able to help.

EXHIBITOR NEWS

Exhibitor News features items provided by exhibitors at the NHF Bleeding Disorders Conference who are also advertising in this publication. The content of these items, as well as the content of their ads, is the sole responsibility of the advertisers. National Hemophilia Foundation and CustomNEWS, Inc., are not responsible for this content.

FACTOR REPLACEMENT REFLECTS THE PROTECTION WITHIN



For people with hemophilia, Factor treatment temporarily replaces what's missing.[1,2] *With a long track record of proven results, Factor treatment works with your body's natural blood clotting process to form a proper clot.*[2-6]

Brought to you by Shire, dedicated to pursuing advancements in hemophilia for more than 70 years.[7]

Stay empowered by the possibilities.

References: 1. Peyvandi F, Garagiola I, Young G. The past and future of haemophilia: diagnosis, treatments, and its complications. *Lancet.* 2016;388:187-197. **2.** Canadian Hemophilia Society. Factor replacement therapy. http://www.hemophilia.ca/en/bleeding-disorders/hemophilia-a-and-b/the-treatment-of-hemophilia/factor-replacement-therapy/. Accessed May 18, 2018. **3.** Franchini M, Mannucci PM. The history of hemophilia. *Semin Thromb Hemost.* 2014;40:571-576. **4.** Hvas AM, Sørensen HT, Norengaard L, Christiansen K, Ingerslev J, Sørensen B. Tranexamic acid combined with recombinant factor VIII increases clot resistance to accelerated fibrinolysis in severe hemophilia A. *J Thromb Haemost.* 2007;5:2408-2414. **5.** Antovic A, Mikovic D, Elezovic I, Zabczyk M, Hutenby K, Antovic JP. Improvement of fibrin clot structure after factor VIII injection in haemophilia A patients treated on demand. *Thromb Haemost.* 2014;111(4):656-661. **6.** Berg JM, Tymoczko JL, Stryer L. Many enzymes are activated by specific proteolytic cleavage. In: *Biochemistry.* 5th ed. New York, NY: WH Freeman; 2002. https://www.ncbi.nlm.nih.gov/books/NBK22589/. Accessed May 18, 2018. **7.** Shire. Shire's 70+ year commitment to the hemophilia community. https://www.shire.com/en/newsroom/2018/january/7sossj. Accessed June 6, 2018.

Shire

Consider the End of the Conference a New Beginning

By Fiona Soltes

So much of the NHF Bleeding Disorders Conference is about the big picture.

But one October 13 session at the 70th annual event brought it back down to scale. **Let's Keep This Going: Staying Connected After NHF2018** talked about the importance of infusing energy at the local level, investing and participating in chapters and in the area community to keep the momentum up all year through.

Dan Levy, whose son Max was the inspiration for Marvel's "Invincible Iron Man" comic, spoke to those gathered about the first time his family attended the national conference a couple of years back, and the high they experienced from the sense of community and support.

"We were on an island by ourselves, until someone from NHF called," Dan Levy said. "It was like, 'Wow. This community is enormous.' We didn't know."

But then, he said, "We went home."

Staying connected throughout the year takes effort. But the payoffs can be great, in a variety of ways. Social media platforms can be used effectively for awareness and fundraising, for example, and connections can be made through hospitals and HTCs. Then there's participation in the local NHF chapter, and organizations such as the National Hemophilia Foundation and the World Federation of Hemophilia. Levy and his family found a way to make it work, and to a rather significant degree; they gained national media attention, and some of the money the family raised now goes to support children with hemophilia in developing nations through Save One Life.

The session was part of the Newly Diagnosed track at the conference, but the lessons within could extend to anyone wanting to increase awareness and community.

Richard Pezzillo, executive director, New England Hemophilia Association, reminded the group that March is Bleeding Disorders Awareness Month, which can be a great jumping off point, and that NHF has educational materials available for distribution.

Families affected by bleeding disorders can raise awareness at their children's schools. In addition, Levy said, a call to the local newspaper or TV station with an idea for a feature might just help increase understanding.

"They'll do it," he said. "They are dying for content. They're always looking for really interesting stories. My son has gone from, 'What's wrong with me?' to a superhero. He's signing autographs and people know who he is." His son's challenges have turned into a positive, he said, and both his family and his community have been changed because of it.

NHF's annual Hemophilia Walks, rebranded this year as Unite for Bleeding Disorders Walks, also provide great opportunities for connection—especially for helping those outside the community get on board.

Those with fresh ideas for outreach, connection and/or services might share them with the closest chapter—as well as volunteer to help get those efforts off the ground. One attendee with a young son, for example, hoped to see parents who had cared for children of their own with bleeding disorders be willing to babysit others. The mother said she's often told she needs to take time for a date night or to care for herself—but has not yet reached the point of being able to trust many others with her child.

"Think about what it is you want when you get back home," Pezzillo told the group. "What spark has been lit?"

It likely will take more than an annual event to keep it burning.

Approved for Hereditary Factor X Deficiency

Coagadex®
Coagulation Factor X (Human)

The first and only treatment specifically for hereditary factor X deficiency

- In clinical studies, COAGADEX was proven effective for on-demand treatment and surgical procedures in patients with hereditary factor X deficiency
- COAGADEX is a high-purity factor X product with factor X content listed on every vial

Visit **www.coagadex.com** for ordering information

Please see the Brief Summary of Prescribing Information on accompanying page.

Indications for COAGADEX

COAGADEX, a plasma-derived blood coagulation factor X concentrate, is indicated in adults and children (aged 12 years and above) with hereditary factor X deficiency for:

- On-demand treatment and control of bleeding episodes
- Perioperative management of bleeding in patients with mild hereditary factor X deficiency

Perioperative management of bleeding in major surgery in patients with moderate and severe hereditary factor X deficiency has not been studied.

Important Safety Information for COAGADEX

COAGADEX is contraindicated in patients with known hypersensitivity to any of the components of the product.

Allergic type hypersensitivity reactions, including anaphylaxis, are possible with COAGADEX. If symptoms occur, patients should discontinue use of the product immediately and contact their physician.

The formation of neutralizing antibodies (inhibitors) to factor X is a possible complication in the management of individuals with factor X deficiency. Carefully monitor patients taking COAGADEX for the development of inhibitors by appropriate clinical observations and laboratory tests.

COAGADEX is made from human plasma and may contain infectious agents, e.g. viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent. No cases of transmission of viral diseases, vCJD or CJD, have been associated with the use of COAGADEX.

In clinical studies, the most common adverse reactions (frequency ≥5% of subjects) with COAGADEX were infusion site erythema, infusion site pain, fatigue and back pain.

Please refer to the COAGADEX Prescribing Information for full prescribing details.

REFERENCE

1. COAGADEX® (Coagulation Factor X, Human) Prescribing Information. Durham, NC: BPL Limited. 2015.

December 2016 US/X/1216/0022

bpl
Bio Products Laboratory
a commitment for life

For medical information queries,
please call **1-866-398-0825**
or email MedInfo@BPL-US.com