Hemogram

WPCNHF

Western Pennsylvania
Chapter of the National
Hemophilia Foundation

Winter 2021
Inside this issue

Fall Program

It was wonderful to have so many families join us on a beautiful fall day for our Fall Program. The event was held over Zoom and began with a fun trivia game, with questions on pop culture and the fall season. Prizes were awarded to the top three high scorers.

Our event sponsors joined us and each of them had the opportunity to speak with our members about their company and products and/or services. Prior to the event, the participants received an event box with materials and/or giveaways from the sponsoring companies.

When the trivia game was over, Janella M. Swartz, RN, Nurse Educator with Octapharma, presented the program Exercise and Your Joints. The session focused on the importance of exercise to maintain joint health. Janella discussed appropriate types of exercise for all age groups and answered questions.

When the event concluded, each family received a gift card to buy lunch.

We thank the following for sponsoring the 2020 Fall Program: BioMarin, CSL Behring, Genentech, the Hemophilia Center of Western PA, Novo Nordisk, Octapharma, Pfizer, Sanofi Genzyme, and Takeda.

Teen Trivia Slam!

By Nicolette C., Teen Group Co-Coordinator

This November, the teen group met for a virtual trivia night. We started off with introductions and welcomed new teens. We also announced that two additional teen coordinators have joined the committee: Ziola J. and Raina S. Then photos from the Halloween costume contest were shared. Shortly after, we answered a few icebreaker questions ranging from sharing what you would do with an extra hour in your day to where you would like to travel. We then discussed future teen events.

Then, the trivia began! We split up into three groups and played multiple rounds of Trivia Slam, hosted by Watson Adventures. This included a movie themed round, a candy round, and even a scavenger hunt round! After we ended, the results were tallied, and the winner was announced: Team Bob's Diner!

After we finished the trivia, the event ended with a zoom group photo.

DESIGNATE UNITED WAY GIFTS TO THE CHAPTER

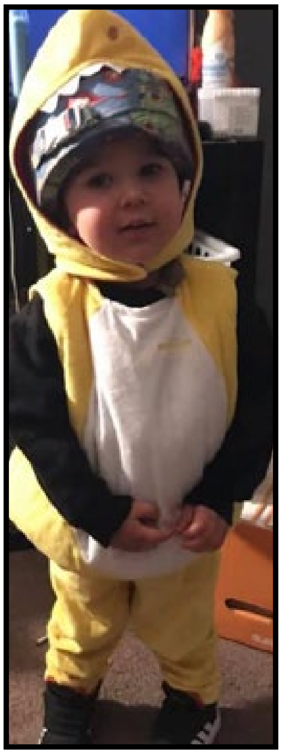
If your company is participating in the United Way campaign, you may designate all or a portion of your gift to the Chapter.

WPCNHF Contributor Agency Code Number is: **83**

Halloween Costume Contest

WPCNHF celebrated Halloween with the Teen Group this year by hosting a Halloween Costume Contest! This contest was open to Chapter members and Campers ages 0-18. Each person who submitted a photo received a prize! Thank you to everyone who submitted a photo. Check out the costumes below!



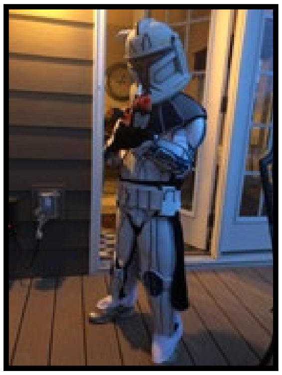




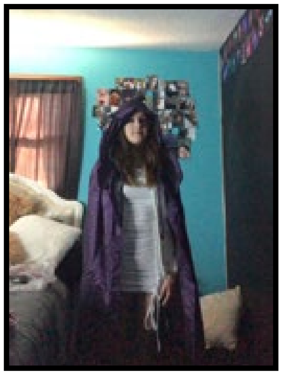
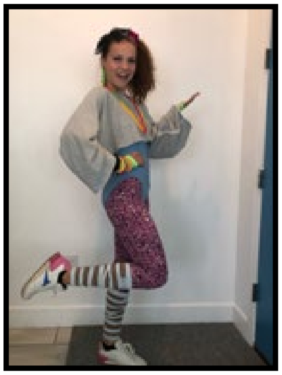









Calendar of Upcoming Events

January 16, 2021
Paint Night
Virtual

January 28, 2021
Drag Queen Bingo
Virtual

February 1-4, 2021
Unite Rally
Virtual

February 25, 2021
Paint Night
Virtual

March 3-5, 2021
NHF Washington Days
Virtual

March 7, 2021
New Parent Network
Virtual

April 17, 2021
World Hemophilia Day
Virtual

July 22, 2021
Annual Meeting
Pittsburgh, PA

August 26-28, 2021
NHF Bleeding Disorders Conference
Virtual

September 18, 2021
Unite for Bleeding Disorders Walk, Run for their Lives 5K, and WPCNHF's 9th Semi-Annual Cornhole Tournament
Allison Park, PA

October 9, 2021
Fall Program
Erie, PA

October 21-24, 2021
HFA Symposium
San Antonio, TX

November 5-7 2021
Education Weekend
Seven Springs, PA

December 4, 2021
Winter Program
Pittsburgh, PA

Visit wpcnhf.org for more information on our upcoming events.

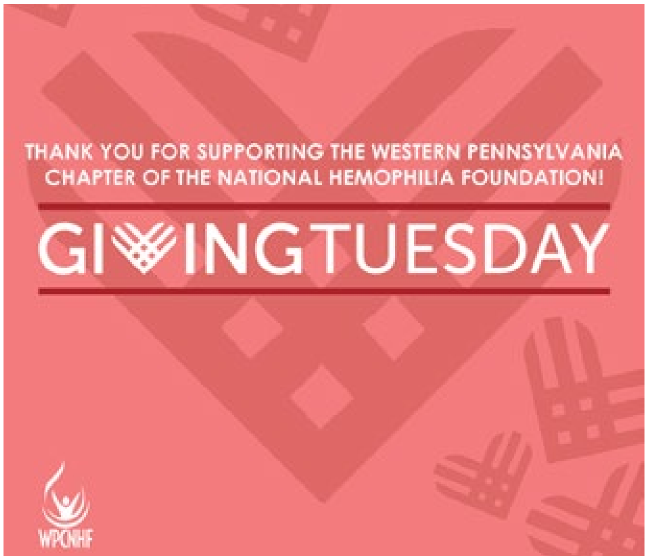
Thursday, January 28, 2021
7:00 PM
$15 PER PERSON

Join us for a night of drag queen bingo from the comfort of your own home! Expect a night of entertainment and prizes! Registration includes Zoom link to watch the performance, a bingo card, and 3 rounds of bingo. Additional cards can be purchased for $2/per card. Zoom link and cards will be emailed to you the night before the event. Register by 6:00 PM on January 28th. This event is for adults.

Our stunning host, Alora Chateaux, is back to help raise critical awareness and funds for the bleeding disorders community of Western Pennsylvania! Additional donations and tips to support our host are greatly appreciated!

Register online at: give.classy.org/wpcnhfbingo

All proceeds support the Western Pennsylvania Chapter of the National Hemophilia Foundation!



Thank you to everyone who supported the Chapter on Giving Tuesday. We raised over $900 dollars to support the bleeding disorders community! Your support will help our members in need with medical bills, transportation to and from appointments, assistance with rent, utilities, groceries, and more.

Thank you for being The Rare Difference by donating and Giving Thanks to our volunteers and supporters. Thank you for being part of our community!

Letter from the Executive Director

Dear Community Members & Friends,

What a year it has been! As we navigate through this health crisis and adapt to the "new normal" of virtual programming and fundraisers, I am so thankful for this community and our ability to adapt and make the best of these challenging times. Always know that the health and wellness of the individuals and families we serve is always our top priority.

We are planning to keep things virtual through the first half of 2021. We have many great educational programs and fun virtual activities planned. I hope you will join and stay connected to the community. Please remember we are all in this together. Do not hesitate to reach out or ask for help if you need it.

The Chapter has increased its ability to provide emergency financial assistance to families with bleeding disorders who have struggled because of the pandemic. Since July, we have been able to assist 120 members, granting over $16,000 through our patient assistance program.

We continued to work hard advocating for the community, and the hemophilia line item in the Pennsylvania state budget was reinstated and funded at the same level as previous years! We are still building our Advocacy Ambassador Program and scheduling virtual meetings with legislators. We hope you will join us in this endeavor as your participation is what will make a real difference in ensuring patient protection and affordable healthcare for the entire bleeding disorders community.

I am truly honored to be leading this organization into 2021. I look forward to continuing to serve this community and I hope to see you at one or many of our upcoming events!

Wishing you good health and happiness in the coming year and always,

Kara Dornish

Kara Dornish
Executive Director

Letter from the President

Hello Chapter Members,

As we look back on this past year, we have had many changes in the way we continue to provide for our members. I would like to thank the Chapter staff for adapting to the needs and changes necessary to still hold valuable programs and to stay connected to the community.

Kara, Janet and Jessica went above and beyond to make sure we still were able to hold programs since everything went virtual. It says a lot about the teamwork coming together.

Looking forward, it seems like this new model for holding events may continue for a while until things can get back to a level where it will be safe for all members to get together again. We appreciate your patience and your continued support of the Chapter.

I would like to extend an invitation to anyone who might be interested in joining the Board of Directors. Please reach out to Kara with your interest and she will be able to guide you in the process of applying. I know there are members out there who would be able to contribute to the board, so I ask that you inquire to get the details.

Kind Regards,

Michael Covert
WPCNHF Board President

Board of Directors

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Mike Covert

Vice President
Brittani Spencer

Secretary
John Yunghans

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Christina Miller

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R. Scott Domowicz
Melinda Perry
Jennifer Smith

Staff

Executive Director
Kara Dornish

Program Director
Janet Barone

Development Director
Jessica Lee

Staff office hours are Monday through Friday from 9 a.m. until 4 p.m. Every attempt will be made to return calls received during regular office hours on the same day.

Mission Statement:

WPCNHF strives to enrich the lives of those with bleeding disorders in Western Pennsylvania and respond to the needs of the community in a dynamic environment.

Hemogram is published quarterly by the Western Pennsylvania Chapter of the National Hemophilia Foundation. The contents of this newsletter may be reproduced freely. The material in this newsletter is provided for your general information only. WPCNHF does not give medical advice or engage in the practice of medicine. WPCNHF under no circumstances recommends particular treatments, and always recommends that you consult your physician or treatment center before pursuing any course of treatment.

Letter from HCWP

Hello to all WPCNHF Members and Your Families,

We hope as winter has set in and the days have shortened, that you have had the chance to snuggle up somewhere warm and take a moment to rest! It certainly has been an odd year, and we must say, in many ways it certainly won't be bad to see the end of it. However, celebrating the holiday season has reminded us that there are also many things we were thankful for this year. We were thankful to be part of such a vibrant community that has so many resources and so much compassion. We were thankful to have the chance to still connect (even if Zoom fatigue is truly a reality). We were thankful that we were able to keep our doors open and keep serving you as best as we have been able. And we have been truly grateful for your patience and adaptability as we have done our best to keep things on track during a remarkable year.

Please remember, if you travel for the holidays (or any other time), to contact us for a travel letter and to make sure you have plenty of factor on hand. (Don't forget, you can leave a message after hours or order online!) Also, if it has been outside of our recommended time-frame for your appointment, please get an appointment scheduled so that we are safely and accurately able to meet your needs. No one wants an emergency, but we do want to be ready if there is one. Keeping up with appointments allows us to be ready to meet your needs.

Unfortunately, in this time that is associated with celebration and plenty, we know that many people are still in very challenging situations. Please remember that we welcome your call, and that the bleeding disorder community is truly a generous community that wants to make sure everyone who is a part of it receives what they need. Winter can be an expensive time. Please reach out to us if things are tough.

We are pleased to share that we continue to improve our telehealth capability, and that we will soon be able to offer a video component through Microsoft Teams for telehealth appointments. Patients can also continue to use the phone only for these appointments.

And as always, please continue to reach out to us with any questions or concerns that you might have about your care or barriers that might keep you from receiving care. Please let us know what would make us better and what has been going well with your care. The more we know, the better we can be.

Sending our best wishes (along with a good dose of optimism for 2021!) to all of you.

The HCWP Staff

The First National Patient Satisfaction Survey of U.S. HTCs

The federally funded hemophilia treatment center (HTC) network, with its model of a multidisciplinary care team (MDT) and regional infrastructure, has proven itself, over several decades, to be well suited to deliver quality, integrated healthcare to bleeding disorders patients across the U.S. While this system has allowed for various surveillance and data collection projects focused on patient demographics, clinical status, and mortality, it has not, until recently, been leveraged to gauge patient satisfaction on a national level.

The authors of a new paper published in the journal Haemophilia posit that patient satisfaction with the delivery of care is an important metric that is associated with treatment adherence and better overall health outcomes. Therefore, a first-of-its kind nationally uniform and comprehensive patient satisfaction survey (PSS) was conducted and made possible via the coordination of the HTC network's robust regional infrastructure. A steering committee made up of three of the network's regional coordinators initiated and managed the PSS. They were guided by performance standards of the hemophilia program of the U.S. Health Resources and Services Administration (HRSA); the agency provides limited funding to support the eight designated regions that make up the HTC network.

The survey was designed to assess patient demographics, their satisfaction with the HTC's core MDT, plus affiliated clinicians, services, and care processes. The MDT core includes the hematologist, nurse, nurse practitioner, social worker, and the physical therapist. In addition, three fundamental HTC services were rated, including shared decision making and care coordination with both the primary doctor and with other specialists/providers. Lastly, five key HTC processes were evaluated for patient satisfaction: timeliness of care, ease of getting needed information, ease of understanding how the HTC clinic staff explained things, time spent with clinic staff, and being treated with respect.

All 138 HTCs that were operating in 2014 were invited to participate in the PSS. The eight regional coordinators of the Network helped facilitate the promotion and dissemination of the survey to HTCs and provided technical assistance to ensure a nationally consistent administration. In February-March 2014 the survey was disseminated to an estimated 28,289 households of patients with which the HTC had a "significant clinical interaction" that same year. The subsequent data collection period through June of 2015, with the University of Colorado serving as coordinating institution for all data collection and aggregation. To make possible comparisons to other populations, regions were collapsed from eight to four standard regions including West, Midwest, South, and Northeast.

The overall participation rate for HTCs was high as 133 or 138 centers (96.4%) opted into the survey. In sum, 5006 individuals who received care from a center in 2014 completed the PSS, representing a 17.7% national response rate. At 29.2%, females represented almost a third of the participants, the majority of which were White, non-Hispanic. A look at participants grouped by age shows a fairly even breakdown amongst the groups, while there were significant differences in participation levels amongst the four geographic regions with 42.1% (2109) from the Midwest, 27.9% (1398) from the Northeast, 19.0% (952) from the West and 10.9% (547) from the Southeast. The majority of the respondents, 3,106 (62%) had hemophilia, 1299 (25.9%) had von Willebrand disease (VWD), and 601 (12.0%) reported diagnosis as "other," "unknown" or did not specify. Overall,

(Continued on page 10)

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. People who use activated prothrombin complex concentrate (aPCC; Feiba®) to treat breakthrough bleeds while taking HEMLIBRA may be at risk of serious side effects related to blood clots.

These serious side effects include:

- **Thrombotic microangiopathy (TMA),** a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs
- **Blood clots (thrombotic events),** which may form in blood vessels in your arm, leg, lung, or head

Please see Brief Summary of Medication Guide on following page for Important Safety Information, including **Serious Side Effects**.

HEMLIBRA®
emicizumab-kxwh
injection for subcutaneous use
150 mg/mL

Medication Guide
HEMLIBRA® (hem-lee-bruh)
(emicizumab-kxwh)
injection, for subcutaneous use

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII (FVIII) and the recommended dose and schedule to use for breakthrough bleed treatment.

HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
  - confusion
  - weakness
  - swelling of arms and legs
  - yellowing of skin and eyes
  - stomach (abdomen) or back pain
  - nausea or vomiting
  - feeling sick
  - decreased urination
- **Blood clots (thrombotic events).** Blood clots may form in blood vessels in your arm, leg, lung, or head. Get medical help right away if you have any of these signs or symptoms of blood clots during or after treatment with HEMLIBRA:
  - swelling in arms or legs
  - pain or redness in your arms or legs
  - shortness of breath
  - chest pain or tightness
  - fast heart rate
  - cough up blood
  - feel faint
  - headache
  - numbness in your face
  - eye pain or swelling
  - trouble seeing

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

See "**What are the possible side effects of HEMLIBRA?**" for more information about side effects.

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

Hemophilia A is a bleeding condition people can be born with where a missing or faulty blood clotting factor (factor VIII) prevents blood from clotting normally.

HEMLIBRA is a therapeutic antibody that bridges clotting factors to help your blood clot.

Before using HEMLIBRA, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if HEMLIBRA may harm your unborn baby. Females who are able to become pregnant should use birth control (contraception) during treatment with HEMLIBRA.
- are breastfeeding or plan to breastfeed. It is not known if HEMLIBRA passes into your breast milk.

Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I use HEMLIBRA?

See the detailed "Instructions for Use" that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes.

- Use HEMLIBRA exactly as prescribed by your healthcare provider.
- **Stop (discontinue) prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis.**
- **You may continue prophylactic use of FVIII for the first week of HEMLIBRA prophylaxis.**
- HEMLIBRA is given as an injection under your skin (subcutaneous injection) by you or a caregiver.
- Your healthcare provider should show you or your caregiver how to prepare, measure, and inject your dose of HEMLIBRA before you inject yourself for the first time.
- Do not attempt to inject yourself or another person unless you have been taught how to do so by a healthcare provider.
- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider.
- You will receive HEMLIBRA 1 time a week for the first four weeks. Then you will receive a maintenance dose as prescribed by your healthcare provider.
- If you miss a dose of HEMLIBRA on your scheduled day, you should give the dose as soon as you remember. You must give the missed dose as soon as possible before the next scheduled dose, and then continue with your normal dosing schedule. **Do not** give two doses on the same day to make up for a missed dose.
- HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

What are the possible side effects of HEMLIBRA?

- See "**What is the most important information I should know about HEMLIBRA?**"

The most common side effects of HEMLIBRA include:

- redness, tenderness, warmth, or itching at the site of injection
- headache
- joint pain

These are not all of the possible side effects of HEMLIBRA.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store HEMLIBRA?

- Store HEMLIBRA in the refrigerator at 36°F to 46°F (2°C to 8°C). Do not freeze.
- Store HEMLIBRA in the original carton to protect the vials from light.
- Do not shake HEMLIBRA.
- If needed, unopened vials of HEMLIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMLIBRA should not be stored out of the refrigerator for more than a total of 7 days or at a temperature greater than 86°F (30°C).
- After HEMLIBRA is transferred from the vial to the syringe, HEMLIBRA should be used right away.
- Throw away (dispose of) any unused HEMLIBRA left in the vial.

Keep HEMLIBRA and all medicines out of the reach of children.

General information about the safe and effective use of HEMLIBRA.

Medicines are sometimes prescribed for purposes other than those listed in a Medication Guide. Do not use HEMLIBRA for a condition for which it was not prescribed. Do not give HEMLIBRA to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about HEMLIBRA that is written for health professionals.

What are the ingredients in HEMLIBRA?

Active ingredient: emicizumab-kxwh

Inactive ingredients: L-arginine, L-histidine, poloxamer 188, and L-aspartic acid.

Manufactured by: Genentech, Inc., A Member of the Roche Group,
1 DNA Way, South San Francisco, CA 94080-4990
U.S. License No. 1048
HEMLIBRA® is a registered trademark of Chugai Pharmaceutical Co., Ltd., Tokyo, Japan

For more information, go to www.HEMLIBRA.com or call 1-866-HEMLIBRA.
This Medication Guide has been approved by the U.S. Food and Drug Administration
Revised: 10/2018

HEMLIBRA®
emicizumab-kxwh | 150 mg/mL
injection for subcutaneous use

Genentech
A Member of the Roche Group

2020 Unite for Bleeding Disorders Walk

This year, we celebrated our 12th Annual Unite for Bleeding Disorders Walk! The Walk was held on Saturday, October 10th and coincided with the National Hemophilia Foundation's (NHF) nationwide Unite Day. While our walk moved virtual and looked slightly different than normal, friends, family, and supporters still came together to raise over $49,000 to support the bleeding disorders community of Western Pennsylvania!

The day started at 10:00 am with the Walk Day Coffee Shop. Our amazing local sponsors had a chance to say hello to the community, and everyone could catch up with friends and family. Five random participants on the Zoom call also won their very own pair of blood drop socks! The Unite presentation and Top Fundraising Awards ceremony began at 11:30 am. The morning came to an end with our Pinwheel ceremony at 11:45 am. A huge thank you to Mason Bobro for the great job he did leading our pinwheel ceremony. Over 30 families and individuals joined the call to Unite for Bleeding Disorders! We also streamed the ceremony live on Facebook.

This day would not have been possible without the support of our many team captains who rallied their runners and walkers and went above and beyond in all that they did. Thank you to all 19 of our Walk Teams! A special shout out to the teams who really went above and beyond and raised $1,000 or more:

Rolling for Ryker – $4,650.00
Conor's Clan – $3,861.86
Red N' Plenty – $2,571.00
Team Jaxson – $2,399.00
Watching River Run – $2,175.00
Cameron's Walking Sticks – $1,920.00
Gino's Gang – $1,495.00
Charlotte's Hemo Hero's – $1,200.00
Aberegg Army – $1,166.00
Maxwell House – $1,045.00

Congratulations to our top fundraising team, Rolling for Ryker, who raised an incredible $4,650! Thank you to the Team Captain of Rolling for Ryker, Mindy Perry-Stern! This year marked the third walk Rolling for Ryker has participated in. Mindy has been an active member of the Chapter ever since her three-year-old son, Ryker, was diagnosed with Hemophilia A in 2017. Last year, she appeared on Wish 99.7 FM with WPCNHF's Executive Director, Kara Dornish, and shared her story with Cris Winter. This year, Mindy's walk team started fundraising way back in November 2019. They hosted a 50/50 raffle and mystery prize raffle and raised over $2,000 in one night! They also hosted a bingo fundraiser at the Red Fox Winery in Hickory, PA in February. The winery generously donated 10% of all their wine sales that night back to the Chapter. We are so thankful for all of Mindy's, and her team's, hard work and dedication to the entire bleeding disorders community.

This year's Top Individual Fundraiser was Tracy Sethman, who individually raised an amazing $2,546. Tracy is the team captain of Team Red N' Plenty. This year was Red N' Plenty's 7th walk! Tracy and her family are affected by von Willebrand Disease and walk to raise awareness for all bleeding disorders. Each year, the team donates raffle items and volunteers to run and sell tickets at the raffle table at the Walk. This year, Tracy put together and donated over 10 baskets! Thank you so much to Tracy and everything she does for the Chapter!

Finally, a big congratulations to the winner of the Awesome John Eyrolles Top Youth Fundraiser Award, Jaxson Baker, who raised an amazing $215! Jaxson is the inspiration for his walk team, Team Jaxson. Jaxson was diagnosed with Hemophilia A when he was seven months old. His parents and big sister, Analise, always join the walk to support him each year! Thank you and congratulations, Jaxson!

Thank you to all of our Factor Club Members who have raised $500 or more for the Unite for Bleeding Disorders Walk! Medals were awarded to the following Factor Club Members:

Brooke Aberegg of Aberegg Army, Cooper Aberegg of Aberegg Army, Josh Baker of Team Jaxson, Jaxson Baker of Team Jaxson, Kelly Baker of Team Jaxson, Janet Barone of Cameron's Walking Sticks, Michael Covert, Cameron Cedeno of Cameron's Walking Sticks, Kara Dornish of Kara's Walkers, Jessica Lee of Team Heart and Sole, Lynda Maxwell of Maxwell House, Conor Nikithser of Conor's Clan, Emily Nikithser of Conor's Clan, Eileen Nikithser of Conor's Clan, Steven Nikithser of Conor's Clan, Melinda Perry of Rolling for Ryker, Michael Perry of Rolling for Ryker, Michelle Perry of Rolling for Ryker, Dawn Rotellini of Gino's Gang, Gino Rotellini of Gino's Gang, Steven Stern of Rolling for Ryker, Ryker Stern of Rolling for Ryker, Robert Sethman of Team Red N' Plenty, Tracy Sethman of Team Red N' Plenty, Jen Werme of Conor's Clan, and Russell Werme of Conor's Clan.

Unite for Bleeding Disorders

Thank you to our Sponsors!

National Presenting Sponsor: Takeda

Official Sponsors: Bayer, briova, Grifols, Optum, Sanofi Genzyme

Partner: accredo

Thank you to our Sponsors! UPMC, Eat'n Park, novo nordisk, 90.5 WESA, The Hemophilia Center of Western Pennsylvania, CSL Behring, My Hero's Sub Shop, Spanos Raymond James, Chick-fil-A, Genentech, American Electric Power, Dunkin', CVS specialty, BioMarin

The Unite for Bleeding Disorders Walk would not have been possible without the extremely generous support of all of you and our National & Local Sponsors.

Our National Sponsors Include: National Presenting Sponsor, Takeda

Our Unite Level Sponsors: Sanofi Genzyme, Bayer, Grifols and Optum

And Our Partner Level Sponsor: Accredo

Our Local Gold Sponsors: UPMC and the Hemophilia Center of Western Pennsylvania

Our Bronze Sponsors: BioMarin, CSL Behring, CVS Specialty, Genentech, and Novo Nordisk

Our Supporter Sponsors: American Electric Power, My Hero's Sub Shop, and Spanos Group of Raymond James

Please take a few minutes to check out their virtual booths by going to bit.ly/wpaunited.

All of the money raised from the Walk stays local to promote patient advocacy, provide educational programming and support groups, and provide financial assistance to families in need. The Chapter offers financial assistance with medical bills, travel to medical appointments, medical devices, medical ID jewelry, and more. The Chapter also uses the money raised to create and increase awareness of bleeding disorders.

Mark your calendar now for next year's Unite for Bleeding Disorders Walk, Run For Their Lives 5k, and Cornhole Tournament taking place at the North Park Pool on Saturday, September 18, 2021!

Upcoming WPCNHF Fundraisers

On Thursday, January 28th, Alora Chateaux is joining us once again for another Drag Queen Bingo Night! Drag Queen Bingo will start at 7:00 pm. Cost is $15 per person and includes Zoom link to watch the performance, a bingo card, and 3 rounds of bingo. Additional cards can be purchased for a $2 per card. Purchase your tickets today at: https://give.classy.org/wpcnhfbingo

WPCNHF's Winning Women Winter Wreath Making Class

Maria Steele-Voms Stein
Women's Group Coordinator

On Saturday, October 24th, the Western PA Chapter's Winning Women's group hosted a Winter Wreath Making Workshop event. The virtual workshop was sponsored by Cottrill's Pharmacy. All of the materials to make the wreaths were thoughtfully put together by Melissa Kendrick, Field Care Coordinator, Cottrill's Pharmacy. During the hands-on wreath making workshop, we were guided step-by-step on creating a beautiful burlap wreath. Wreaths created by attendees during the event were donated to the Chapter's Take A Bough fundraising event. Thank you to all who participated and so kindly donated your beautiful creations back to the Chapter!

The WPCNHF Winning Women's group is a great opportunity for the women of our Chapter to network with other women in our community, learn about issues related to bleeding disorder management and to have a lot of FUN! I hope to see you at an upcoming WPCNHF Winning Women's group or other Chapter event soon.

The Chapter thanks Cottrill's Pharmacy, Inc. for sponsoring this program.

This wreath was made with care by Michaelia A.

The First National Patient Satisfaction Survey of U.S. HTCs

(Continued from page 5)

those with a severe hemophilia and type 3 severe von Willebrand disease represented 29.4%, of respondents, while those with a moderate bleeding disorder, including VWD type 2 or moderate hemophilia accounted for 17.8% of participants. Those with a mild bleeding disorder (VWD type 1 or mild hemophilia) comprised the largest group at 32.8%.

By virtually all measures, results of the PSS suggest consistently high levels of satisfaction with HTCs amongst the more than 5,000 respondents. Overall, 94.2%-97.9% reporting responded that they were 'always' or 'usually' (A/U) satisfied with the overall care they received at their center. Participants also rated highly their satisfaction with members of their HTC's core MDT, including the hematologist, nurses, nurse practitioners, social workers, and physical therapists (PTs). A national breakdown by member showed that 97.3% of respondents were A/U satisfied with the hematologist, 97.0% with the HTC nurse and nurse practitioner (combined), 95.1% with the social worker and 95.6% with the PT.

HTC care processes considered integral to PSS also scored very well nationally, with more than 95% of respondent's A/U satisfied with each of the five processes. These included timeliness of care (94.9%); ease of getting needed information (95.0%); ease of understanding how the HTC clinic staff explained things (97.3%); time spent with clinic staff (97.0%); and being treated with respect (98.0%). On a regional level, A/U satisfaction for each of the five care processes was at least 91.2%.

The survey also reflected well on transition issues nationally. Of respondents aged 12-17 years, 90.2% reported being A/U satisfied with how their HTC talked about how to care for their bleeding disorder as they became adults. Similarly, 92.8% of adolescents were A/U satisfied with how their HTC encouraged them to become more independent in managing their bleeding disorder.

The authors point to this initiative's success as a "proof of concept" in the far-reaching utility of a regional infrastructure to deliver meaning and impactful national assessments now and in the future.

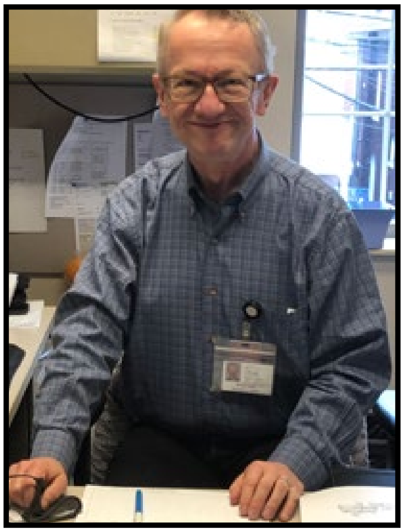
"This HTC PSS initiative provides new national data, reducing evidence gaps in quantifying the extent to which patients value the different healthcare professionals on the integrated HTC team, HTC services, processes and overall care. These high levels of patient satisfaction were articulated regardless of patient diagnoses, severity of disease, gender, race or ethnicity, or geographic location, and pose several implications, "explained the authors. "First, these data indicate that patients highly value the HTC multidisciplinary team approach."

While the authors do acknowledge limitations, including an imbalance in regional representation, the take home message remains the same; there exists a strong correlation between patient satisfaction and quality of care. Further, such PSS data may be employed in various advocacy efforts, to engage payers, and ensure the viability of the HTC Network.

"The high level of patient satisfaction documented in this inaugural national survey of the US HTC Network's ambulatory services has several important policy implications. Specifically, in the United States, access to HTC care must be guaranteed," conclude the authors. "All payers must include HTCs in their networks to maintain high-quality patient care."

Riske B, Shearer R, Baker J. Patient Satisfaction with US Hemophilia Treatment Center Care, Teams and Services: The First National Survey. Haemophilia. 23 October 2020.

Getting to Know HCWP Staff



Bruce Haas
HCWP Genetic Counselor

A man of many credentials (MS, LCGC, (CG)ASCP), Bruce is a wonderful addition to the HCWP team! Here is some info about him so you can get to know him better.

Birthplace: Wisconsin Rapids, Wisconsin

First job: Lab technologist researching placental dysfunction in mice

Accomplishment you're proudest of: Having trained many genetic counselors

What three words describe you best? Patient, attentive, perceptive

Dream vacation: Amsterdam

Things you can't do without: Diet Coke and cheese

Person you'd most like to have dinner with: Meryl Streep

Movie you could see anytime: Chariots of Fire

TV show you try not to miss: The national news

Three things that can always be found in your refrigerator: Diet Coke, cheese, and yogurt

Secret vice: I sing Broadway show tunes to my cats- one of whom hides under the bed.

Who would play you in the movies? Judge Reinhold

Your pet peeve about Pittsburgh: Not here long enough to have one…

People may be surprised to know that: I snuck Cat Stevens, CSNY, and Elton John albums into the USSR in 1977 and left them there with new acquaintances.

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antihemophilic factor
(recombinant) PEGylated-aucl
LET'S GO

BAYER

Jivi® Extension Study

Explore the study design and see the safety and efficacy data from patients who were part of the study.

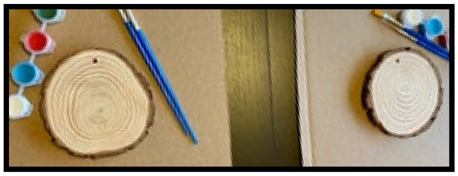
▶ **Dive in at *JiviExtensionStudy.com***

Winter Program

Although we would have loved nothing more than to gather in person and spend time with Chapter families, we did the next best thing and gathered over Zoom! We really enjoyed seeing everyone.

Our Winter Program, which was held on Sunday, December 6, began with a challenging trivia game and prizes were awarded to the top three high scorers. Prior to the event, participants were mailed an event box which contained materials and/or giveaways from sponsors. All sponsors had an opportunity to speak during the event and provide information about their companies.

The event wrapped up with a painting activity. Prior to the event, art supplies were mailed to everyone who registered. Mickayla Poland, local artist, joined us for the event and instructed us step-by-step in painting a winter scene on a wooden disc. The painted wood ornaments turned out beautiful!



We thank the following for sponsoring this event: BioMarin, the Colburn-Keenan Foundation, CSL Behring, CVS Specialty, Genentech, the Hemophilia Center of Western PA, Novo Nordisk, and Takeda.

Virtual 8th Annual Take A Bough is Tree-Mendous!

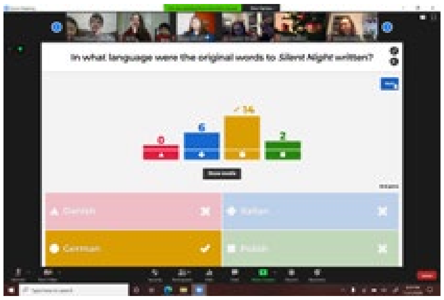
The 8th Annual Take A Bough went virtual this year and incorporated a winter themed holiday trivia game and mixology night to keep everyone connected even while apart.

Thanks to the support from our sponsors, donors, and patrons, we raised over $11,000! We are extremely grateful for everyone who participated in Take A Bough this year and made it a huge success. This year's auction opened online on November 16th, one week prior to the virtual event that took place over Zoom on November 21st. This year, instead of a wine tasting, local wineries were invited to join our call and they taught us how to make drinks using their wine and cider.

Arsenal Cider joined us again this year and taught us how to make a Mulled Arsenal Cider using their Archibalds Ado Cider. Arsenal Cider generously donated 10% of the bottle or growler sales of Archibalds Ado for Take A Bough back to WPCNHF! Thank you to Michelle and Arsenal Cider for joining us!

Heather, from One Hope Winery, also joined Take A Bough this year and taught us how to make a Harvest Sangria. 10% of all wine sales between November 11th and November 25th went back to WPCNHF! Thank you so much for your support, Heather!

Planning and preparation for this event began months before November 21st. Our Co-chairs, Mindy Perry-Stern and Maria


In what language were the original words to *Silent Night* written?

Steele-Voms Stein, helped gather trees, decorations, gifts, and raffle items leading up to the event. While volunteers were unable to come to the Chapter office to help decorate, we are so thankful for our community members who decorated trees, wreaths, and centerpieces at their homes and dropped them off or mailed them to us. In total, we had five full-size trees, five tabletop trees, 34 wreaths (14 that were made by our Winning Women's Group), and 12 centerpieces. Thank you to the Shoemaker family for donating a brand-new Kitchen Aid Professional 5 Plus mixer to our auction! A special thank you to Laureen Temple for making and donating 9 beautiful centerpieces, and many thanks to Manny Lopez of Cottrill's Pharmacy for donating a pair of hand carved end tables to our auction!

The virtual Final Night Event started at 7:00 pm on Zoom. After Arsenal Cider's presentation, we started our Winter Trivia game. There were three rounds in the game and the first place winner received a Kindle, the second place winner received an Echo Dot Smart Speaker with Alexa and LIFX Smart Bulb, and the third place winner received a $15 Amazon gift card. Congratulations to our winners, Emily N. Orest M., and Jim C.! A huge thank you to John and Christina Yunghans for emceeing our trivia night!

In between trivia questions, John and Christina highlighted various items up for bid in our auction and passed it over to Mindy and Michael to pull numbers for our reverse raffle. Tickets were sold for $10 each and participants had the chance to win $250! Mindy sold all 100 tickets in just one day! The winner of the reverse raffle donated $150 back to the Chapter, which made the total raised from the reverse raffle $900 for WPCNHF! Thank you again to Mindy and her dad, Michael Perry, for hosting this raffle for us!

The auction and virtual event ended at 9 p.m. Participants were able to pay their invoices online or on the Handbid app on their phone and could arrange to pick up their items at the Chapter office starting Monday, November 23rd. The Western Pennsylvania Chapter of the National Hemophilia Foundation would like to thank Mindy and Maria for co-chairing the event, Hope Winery and Arsenal Cider House, and the many community members who donated their time and items to the auction. We would also like to thank our sponsors:

Our Silver Sponsor: The Hemophilia Center of Western Pennsylvania

Our Bronze Sponsor: BioMarin

Our Supporting Sponsor: Spark Therapeutics

Our Patron Sponsors: CSL Behring and Armstrong

Our Media Sponsor: WYEP 91.3

Successful fundraisers like Take A Bough allow the Chapter to continue to carry out the mission of enriching the lives of those with bleeding disorders in Western Pennsylvania and respond to the needs of the community in a dynamic environment.


Take A Bough

Thank You to Our 2020 Take A Bough Sponsors!

WPCNHF

Silver Sponsor

The Hemophilia Center of Western Pennsylvania

Bronze Sponsor

BioMarin

Supporting Sponsor

Spark

Patron Sponsor

CSL Behring Biotherapies for Life

Armstrong

Media Sponsor

wyep 91.3

Zoo Lights Event

On Friday, December 11, WPCNHF invited our members to visit the Pittsburgh Zoo and PPG Aquarium Zoo Lights! We were able to provide tickets to 18 families who enjoyed themed light displays, classic characters, and carols along the way! WPCNHF would like to thank the Colburn Keenan Foundation for supporting this event!

Colburn Keenan Foundation, Inc.

Advocacy Update

We are excited to let you know the Hemophilia SNF Access Act was included in the end-of-year omnibus federal spending and economic relief package passed by Congress on December 21, 2020 (HR 133, The Consolidated Appropriations Act, 2021). The bill was a top talking point for advocates who participated in NHF's Washington Days on February 28th, as well as the Hemophilia Alliance Virtual Hill Days, which were held during the last week of July. Thanks to all of you who attended meetings, wrote letters, and made phone calls to federal legislators throughout the year asking for their support. Your hard work has paid off!

The Annual PA Advocacy Stakeholders Meeting took place virtually on Tuesday, November 17, 2020. The Western and Eastern PA Chapters came together to hold this meeting. Kara and Curt gave Chapter updates, Miriam Goldstein of HFA and Nathan Schafer of NHF presented on federal issues, and Erik Ross and Kerry Lange of Milliron and Goodman informed the group of state issues. We were joined by representatives from the 7 HTCS in Pennsylvania and Chapter Advocacy Ambassadors. We are looking forward to building our Advocacy Ambassador Program and continuing the strong partnership with the Eastern PA Chapter.

THE NATIONAL HEMOPHILIA FOUNDATION WILL BE HOSTING ITS ANNUAL WASHINGTON DAYS ADVOCACY EVENT, VIRTUALLY, MARCH 1-5, 2021

The National Hemophilia Foundation's Washington Days this year is virtual! **Training will be held Monday, March 1st** from 3pm-6pm. Legislative meetings will be held **Wednesday, March 3rd and Thursday, March 4th.** You must attend the training in order to attend the legislative meetings. There will also be a state/local advocacy training on Friday, March 5 from 12:00 - 2:00PM.

If you would like to join us in advocating for the bleeding disorders community, you can sign up by emailing rsvp@wpcnhf.org by Monday, February 1st.

On November 23, 2020, Governor Tom Wolf signed the state budget for the remainder of the fiscal year into law. Senate Bill 1350 is the Supplemental Appropriations Bill which appropriates dollars for the remaining 7 months of the 2020-21 budget. The Hemophilia program line item remained fully funded at $559,00 (7/12). In addition, HB 2536 was signed into law which is the Fiscal Code Bill. This important companion bill implements the state budget and is the roadmap for how the appropriated budget dollars are spent. The language remained the same stating "FUNDS APPROPRIATED FOR HEMOPHILIA SERVICES SHALL BE DISTRIBUTED TO GRANTEES IN THE SAME PROPORTION AS DISTRIBUTED IN FISCAL YEAR 2019-2020." Our success with the line item and the language of the fiscal code bill is due to the Chapter's ongoing legislative meetings and the important relationships we have built with these legislators.

Become an Advocacy Ambassador
Advocacy Ambassadors are volunteers in a leadership position responsible for helping to establish and build a strong grassroots network of bleeding disorders advocates within Pennsylvania. The goal is to increase awareness of Hemophilia, von Willebrand disease, and other factor deficiencies and the challenges persons affected and their families face. Ambassadors serve as liaisons between the Pennsylvania Hemophilia Foundations and the public, state agencies and officials working towards improving the quality of life of individuals and their families who are affected by bleeding disorders. If you would like to become an Advocacy Ambassador of WPCNHF please email Kara at kara@wpcnhf.org or Janet at janet@wpcnhf.org for more information.

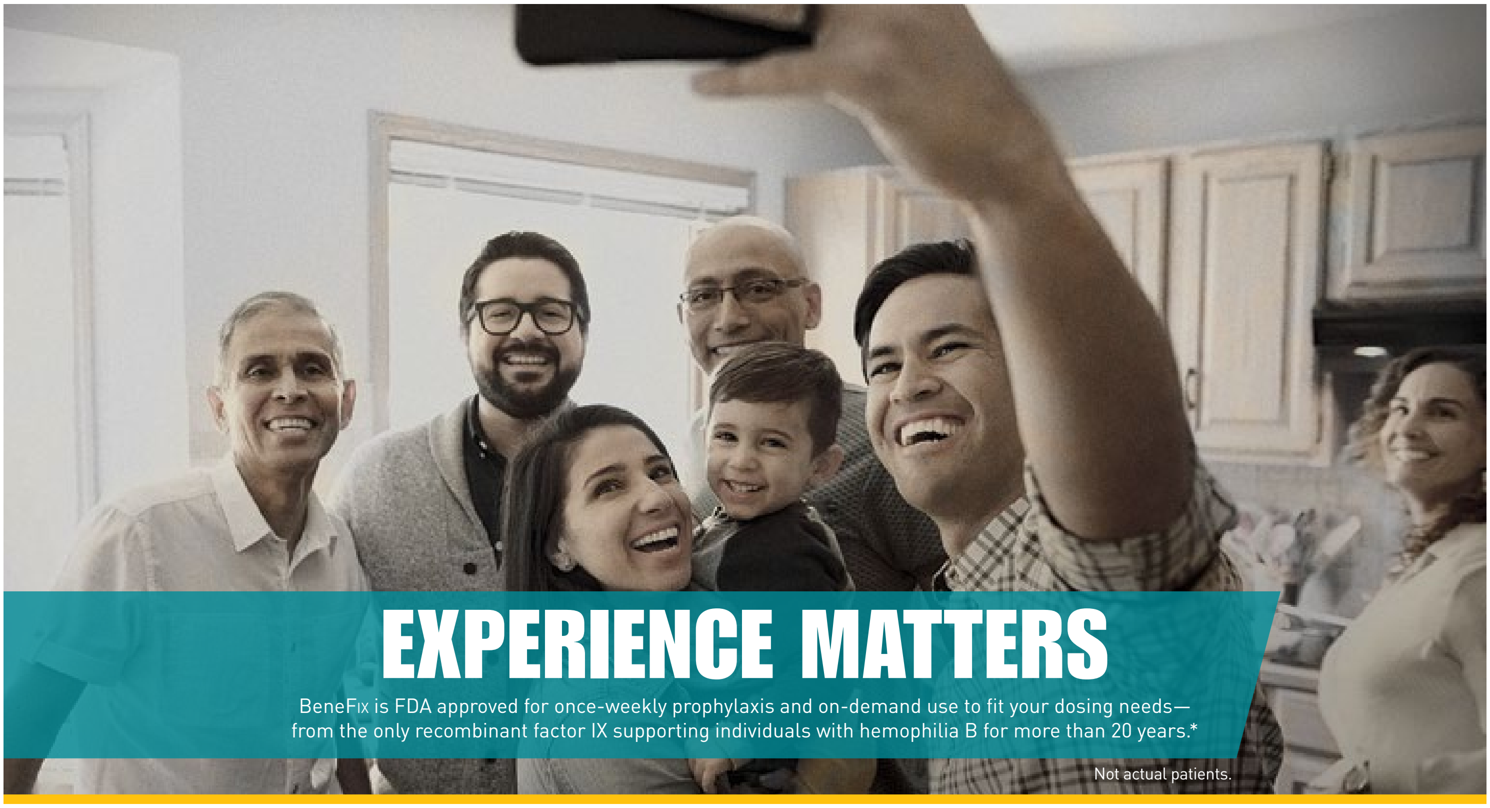
New Parent Network

Ever since the pandemic hit, families have been spending more time at home and indoors; and with colder weather approaching, that is not likely to change. So, we invited our New Parent Network families and a few experienced, mentor families to join us for a Zoom event to share their ideas for keeping their kiddos with bleeding disorders safe indoors, while keeping their sanity!

The event was held on Sunday, November 22. We enjoyed the participation from parents and children alike! The families shared what their favorite activities are to burn off energy indoors; parents shared child-proofing hacks, and the families also shared what makes them happy when they need time to themselves. After the event, the families received gift cards to buy dinner.

The New Parent Network events are for families who have a young child with a bleeding diagnosis. If you have a child, newborn to age 7, who has a bleeding diagnosis and you would like to attend these events and/or be connected with another parent of child with a bleeding diagnosis, please reach out to either Janet (janet@wpcnhf.org / 724-741-6160) or Kathaleen (kschnur@vitalant.org / 412-209-7267).

We thank the following for sponsoring the 2020 New Parent Network series of events: CSL Behring, CVS Specialty, the Hemophilia Center of Western PA, Novo Nordisk, Sanofi Genzyme, and Takeda.



EXPERIENCE MATTERS

BeneFIX is FDA approved for once-weekly prophylaxis and on-demand use to fit your dosing needs—from the only recombinant factor IX supporting individuals with hemophilia B for more than 20 years.*

Not actual patients.

- **More than 20 years* of experience—**the first recombinant treatment for individuals with hemophilia B
- **Dosing options to meet your needs—**for once-weekly prophylaxis and on-demand use
- Designed with viral safety in mind. More than 150 quality control tests are done on each batch of BeneFIX
- The convenience of the BeneFIX Rapid Reconstitution (R2) Kit with a range of vial sizes

What Is BeneFIX?

BeneFIX, Coagulation Factor IX (Recombinant), is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Your doctor might also give you BeneFIX before surgical procedures.

BeneFIX is **NOT** used to treat hemophilia A.

ASK YOUR DOCTOR WHICH BENEFIX DOSING OPTIONS MAY BE RIGHT FOR YOU

Important Safety Information

- BeneFIX is contraindicated in patients who have manifested life-threatening, immediate hypersensitivity reactions, including anaphylaxis, to the product or its components, including hamster protein.
- Call your health care provider right away if your bleeding is not controlled after using BeneFIX.
- Allergic reactions may occur with BeneFIX. Call your health care provider or get emergency treatment right away if you have any of the following symptoms: wheezing, difficulty breathing, chest tightness, your lips and gums turning blue, fast heartbeat, facial swelling, faintness, rash, or hives.
- Your body can make antibodies, called "inhibitors," which may stop BeneFIX from working properly.
- If you have risk factors for developing blood clots, such as a venous catheter through which BeneFIX is given by continuous infusion, BeneFIX may increase the risk of abnormal blood clots. The safety and efficacy of BeneFIX administration by continuous infusion have not been established.
- Some common side effects of BeneFIX are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness, and rash.

Please see the Brief Summary for BeneFIX on the next page.

YOUR EXPERIENCE 20+ YEARS* MATTERS

BeneFIX®
Coagulation Factor IX (Recombinant)
Room Temperature Storage

*BeneFIX was approved February 11, 1997.

PP-BEN-USA-0471

July 2020

Pfizer

BeneFIX®

Coagulation Factor IX (Recombinant)

Room Temperature Storage

℞ only

Brief Summary

See package insert for full Prescribing Information. This product's label may have been updated. For further product information and current package insert, please visit www.Pfizer.com or call our medical communications department toll-free at 1-800-438-1985.

Please read this Patient Information carefully before using BeneFIX and each time you get a refill. There may be new information. This brief summary does not take the place of talking with your doctor about your medical problems or your treatment.

What is BeneFIX?

BeneFIX is an injectable medicine that is used to help control and prevent bleeding in people with hemophilia B. Hemophilia B is also called congenital factor IX deficiency or Christmas disease. Your doctor might also give you BeneFIX before surgical procedures.

BeneFIX is **NOT** used to treat hemophilia A.

What should I tell my doctor before using BeneFIX?

Tell your doctor and pharmacist about all of the medicines you take, including all prescription and non-prescription medicines, such as over-the-counter medicines, supplements, or herbal medicines.

Tell your doctor about all of your medical conditions, including if you:

- have any allergies, including allergies to hamsters.
- are pregnant or planning to become pregnant. It is not known if BeneFIX may harm your unborn baby.
- are breastfeeding. It is not known if BeneFIX passes into the milk and if it can harm your baby.

How should I infuse BeneFIX?

The initial administrations of BeneFIX should be administered under proper medical supervision, where proper medical care for severe allergic reactions could be provided.

See the step-by-step instructions for infusing in the complete patient labeling.

You should always follow the specific instructions given by your doctor. If you are unsure of the procedures, please call your doctor or pharmacist before using.

Call your doctor right away if bleeding is not controlled after using BeneFIX.

Your doctor will prescribe the dose that you should take.

Your doctor may need to test your blood from time to time.

BeneFIX should not be administered by continuous infusion.

What if I take too much BeneFIX?

Call your doctor if you take too much BeneFIX.

What are the possible side effects of BeneFIX?

Allergic reactions may occur with BeneFIX. Call your doctor or get emergency treatment right away if you have any of the following symptoms:

wheezing
difficulty breathing
chest tightness
turning blue
(look at lips and gums)
fast heartbeat
swelling of the face
faintness
rash
hives

Your body can also make antibodies, called "inhibitors," against BeneFIX, which may stop BeneFIX from working properly.

Some common side effects of BeneFIX are fever, cough, nausea, injection site reaction, injection site pain, headache, dizziness and rash.

BeneFIX may increase the risk of thromboembolism (abnormal blood clots) in your body if you have risk factors for developing blood clots, including an indwelling venous catheter through which BeneFIX is given by continuous infusion. There have been reports of severe blood clotting events, including life-threatening blood clots in critically ill neonates, while receiving continuous-infusion BeneFIX through a central venous catheter. The safety and efficacy of BeneFIX administration by continuous infusion have not been established.

These are not all the possible side effects of BeneFIX.

Tell your doctor about any side effect that bothers you or that does not go away.

How should I store BeneFIX?

DO NOT FREEZE the BeneFIX kit. The BeneFIX kit can be stored at room temperature (below 86°F) or under refrigeration. Throw away any unused BeneFIX and diluent after the expiration date indicated on the label.

Freezing should be avoided to prevent damage to the pre-filled diluent syringe.

BeneFIX does not contain a preservative. After reconstituting BeneFIX, you can store it at room temperature for up to 3 hours. If you have not used it in 3 hours, throw it away.

Do not use BeneFIX if the reconstituted solution is not clear and colorless.

What else should I know about BeneFIX?

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

If you would like more information, talk with your doctor. You can ask your doctor or pharmacist for information about BeneFIX that was written for healthcare professionals.

This brief summary is based on BeneFIX® [Coagulation Factor IX (Recombinant)] Prescribing Information LAB-0464-12.0, revised June 2020.

July 2020

Pfizer

Member Spotlight: Meet Donald Miller

Soon after we published an article recapping a gene therapy educational program we held, Don Miller reached out to us to comment on information in the article and we are so glad he did! We were intrigued with Don's experience with gene therapy and wanted to get to know him better and learn more about the research that had taken place years ago. We hope you enjoy getting to know Don as much as we have.

Don was born on Christmas Eve, in 1948. His father worked as a sewing machine mechanic for several factories around Boswell, PA, trying to make enough money in order to continue being a dairy farmer. He gave up farming within a decade and made umpteen times as much money operating a dragline on the nightshift, in a coal mine. His Mother was a Chicago woman his dad met whilst stationed in the Great Lakes during the war.

Around 18 months of age, Don climbed out of his crib and wound up bruised from the waist up. Don and his mother took the B&O Railroad from Rockwood, PA to John Hopkins in Baltimore, where he received the diagnosis of Hemophilia. According to his mother, there had been an obvious problem since circumcision; however, there was no prior family history. No other family members have since been diagnosed. One of his sisters had scientifically tested negative to be a carrier and his other sister had three sons and five grandsons (none had Hemophilia) and the family considered that sufficient testing! 😊

One of Don's earliest memories, around the age of six or seven, is getting off the bus across the street from Montefiore Hospital, in Pittsburgh, for appointments when the Hemophilia Center was in the Central Blood Bank. Don also recalls an ambulance ride with his parents to Children's Hospital after he coughed a flap of skin loose in his throat. That incident resulted in a one-week hospitalization.

During hemorrhages in his earlier years, Don spent a lot of time lying in bed with ice packs and elastic bandages, and he learned self-hypnosis to block most of the pain. At the time, whole blood was the only treatment, using large, 12-gauge needles.

In 1960, Don was diagnosed at Children's Hospital with polyps of the intestines, resulting in a small intestine resection. He later learned that he was given only a 10% chance of surviving the operation & healing. He spent five weeks in the hospital. At this time, Cryoprecipitate (a substance rich in factor VIII, that is derived from thawing fresh frozen plasma) was available, and by the time he left the hospital, he had gone through well over 100 pints of blood and cryoprecipitate. The women who worked in the sewing factories held blood drives to replace the blood so his parents would not need to pay for it. The replacement ratio was two pints of blood for each of Cryoprecipitate. During his hospital stay, he ate a diet consisting of mashed potatoes, creamed hamburger,

(Continued on page 19)

SAY HELLO TO JAMES

He has hemophilia A and has gone through two major surgeries while keeping to his factor regimen with the support of his hemophilia care team

"RECOVERY WAS TOUGH, BUT I LEARNED I HAD MORE SUPPORT THAN *I THOUGHT POSSIBLE.*"

HELLO FACTOR

Read stories like James' in *Hello Factor* magazine:
BleedingDisorders.com

Takeda

Member Spotlight: Meet Donald Miller

(Continued from page 17)

and peas. He was warned to be careful of his eating habits or to expect a recurrence. “With my usual obedience—none—I have enjoyed red meat, raw fruit & vegetables, popcorn, Habanero peppers—all the “mild” consumables,” says Don, nearly 61 years since receiving that warning!

Born with his left leg one-half inch shorter than the right, Don developed a target joint in his left knee early on and eventually his knee was frozen at an angle. A slip resulted in five weeks in a half-body-cast to mend a broken left femur. He used a wheelchair for several years and then around the age of 11, he spent 6 weeks in a half-cast at Children’s Hospital to straighten his left knee. For the next 6 years he wore a full leg brace, eventually working up to about 75% mobility in his knee.

Don went to school for the first time when he entered the eighth grade. He was home-tutored for the first 7 years, half of that time he spent in a wheelchair. After his procedure at Children’s Hospital to straighten his leg, he attended Boswell High School. Don was a teacher’s pet, not because of his condition, but because he did all the homework, made mostly A’s on tests, and had his hand up quickly. Don says, “…that lasted through graduation as did the annual taunts of ‘Pegleg,’ and ‘Brains.’ I handled the first by demonstrating a dozen one-legged (right leg) deep knee bends, then challenging my opponent. He would fall over after maybe 3-4, his friends would laugh at him, and I was okay for the year. Not much you can do about the second: Nerds is Nerds.”

According to Don, pain is mostly forgettable, or no woman would have multiple children. He just accepted that he should not be too adventurous. During his childhood, he spent a lot of time in bed, but life on his parent’s farm was not bad. Even when times were hard, they still had plenty to eat. They ate beef frequently and Don believes that an enzyme found in beef, which promotes clotting, may have helped. The family also ate fried pumpkin blossoms and Morel mushrooms by the peck, long before they became trendy. His father made use of the many Silver Maple trees on their property and made their own real maple syrup.

Don met his wife, Barb, during orientation at Johnston Pitt, when he went to apply for work/study in the library. 55 years later he still sees her as the tall, slim brunette in tight jeans, gingham shirt, and bandana. Only one term at the Main campus (for Barb), some short-term business trips, and hospitalizations have separated them in all these years. Don likes to tell people that Barb has hung around because she got to stick needles in him!

Over the four years of college, while Don was earning his Bachelor of Science degree in Physics, he had spontaneous bleeding through his left ear drum, left eye, elbows, right shoulder, and kidneys. He says that that was enough to convince him that Grad School would be a death sentence, as he noticed stress was leading to him having major bleeds before major tests, so he decided not to continue with the Doctoral program.

Upon graduation, Don worked with digital imaging in surgery with one of the first men in the field; he also worked on X-ray research, worked as an X-ray Safety Officer, worked as a computer consultant, and spent six years in charge of Pitt’s Mathematics Library. He worked until the age of 30, when he was given three months to live. Don had a long history with hepatitis. He was first diagnosed with Hepatitis A around the age of 9 and was soon diagnosed with Hepatitis B and then later with Hepatitis C. The hepatologist told him he might stretch out his remaining years if he cut back on stress. Since Barb had a good job, their property was almost paid off, and they had no children, Don was able to quit his job and rest until the affects of hepatitis subsided. He has outlived the hepatologist who broke the news to him and has lasted another 41 years so far!

Fast-forward 20 years: The year was 1999 and Don was 50 years old. Dr. Ragni announced the first gene trials and Don volunteered the next day. With no children to consider, and Barb having her own career, he had no hesitation signing up for the trial.

As recalled by Don, the trial was three injections of mutated HIV genes, followed by blood draws, hourly, then daily, then weekly, for maybe 6 months. The highest measured level of factor VIII in Don was 1%, once. The animal trials had led the researchers to believe that it should have been 10-20%, so, it was considered a failure. The results were in line with results of other organizations that were involved with this research.

Today, Don and Barb are living on their 150-acre farm and Don helps the rancher who’s leasing to buy it from them. He also keeps busy dismantling reusable parts from non-working machinery, mowing the three acres closest to the house, directing a worker on the property, and editing books that his wife writes.

When asked if he has any advice for someone who might consider participating in a trial, Don’s opinion is that clinical trials can be dangerous, but no more so than trying any medicine or food you’ve never had before, acknowledging that different bodies react to the same stimulus in different ways (think of food allergies). He feels that the decision on whether or not to participate is based in your circumstances. He was comfortable participating because Barb was in a profitable field and they, by choice, had no children. He says, “don’t let the fear of the unknown cripple you. You could die in a wreck tomorrow, but does that stop you from driving to work or play?”

We thank Don for sharing the story of his life with a bleeding disorder. He has lived through the advancement of treatment options from whole blood, to fresh frozen plasma, to cryoprecipitate, to freeze-dried powdered factor concentrate that enabled home infusion. So much has changed regarding treatment options in the past 70+ years. Now, there are several longer-lasting products available; plus,clinical trials for numerous new therapies, including gene therapy, are underway; and FDA approval for gene therapy in the near future is looking promising.

If you are considering participating in a trial, talk with your health care provider about risks and potential benefits and then discuss them with your family and friends. Visit clinicaltrials.gov for a list of questions you should ask to help you make an informed decision.

Each year does end and a new one begins. May this coming new year be one that leaves your worries behind, and one that gives you encouragement and hope.

From all of us at the HCWP— we are looking forward to serving you in 2021. We thank you for trusting us to be a part of your care, and wish the best for you and yours in the new year!

Western Pennsylvania Chapter of the National Hemophilia Foundation
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Cranberry Township, PA 16066
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