



THE ARTERY

WINTER 2022



THIS ISSUE

06 NATIONAL WOMEN
AND TEEN GIRLS
CONFERENCE

13 PROTECT YOUR
MEDICAID
COVERAGE





Susan Fenters Lerch interacting at the Women and Teen Girls National Conference

DEAR HFM FRIENDS AND FAMILY,

With 2023 quickly approaching please know how grateful I am, together with HFM's team, to our community for your continued support, engagement with programs, as well as your willingness to share ideas. We appreciate opportunities to work together sharing information, education, and available services via HFM and related organizations.

Following our full summer, we have continued with a myriad of events this fall. Programs have included a variety of Community Nights, Dinner Talks, Ruby Connection virtual gatherings, camp alumni's Old Beagle, Men's virtual support group, Michigan All Copays Count advocacy efforts – including a legislative day in Lansing—HFM's National Conference for Women and Teen Girls with Hemophilia and Rare Factor Deficiencies, Unite Walks in both west and southeast Michigan, youth retreats, bi-monthly zoom meetings with our federal Great Lakes Region hemophilia treatment centers, the in-person Regional HTC Network meeting, as well as various year-end gatherings. The quality, creativity, and commitment of our HFM team is exceptional, and I hope you join me in appreciating HFM's collective efforts.

Though we will continue many virtual activities, we plan to reinstate more in-person and hybrid events and programs as we end 2022 and move into the New Year.

In collaboration with HFM's Board of Directors and staff please find our updated COVID policy:

HFM continues to recommend vaccinations/boosters and use of masks as preventative measures against the spread of COVID-19. However, we will no longer require proof of vaccination to attend in-person events. Masking, temperature checks, and other protocols may take place based on current COVID data and can vary from event to event. This change in policy is based on our current understanding of the severity and prevalence of Covid-19 and may be adjusted in the future as we deem necessary.

Fingers crossed we'll be able to spend more time with one-another in-person as we move forward!

Another area that is achieving forward momentum is gene therapy treatment for adults with hemophilia A and B. Gene therapy products have been approved in Europe and there is an expectation there will be approval coming in the United States. Our Great Lakes Region HTC Network is considering how we will best offer access to gene therapy for interested and eligible patients. We encourage you to visit information offered by the National Hemophilia Foundation, Hemophilia Federation of America, as well as discussions with your treatment center. We will provide access to informational updates regarding gene therapy and other treatments, though HFM does not recommend any specific treatment products. For more information, visit: www.hemophilia.org/bleeding-disorders-a-z/treatment/future-therapies/resource-guide

As 2022 comes to a close and we look to the future I wish you and yours much love and light.

With gratitude,

Susan Fenters Lerch
HFM Executive Director
Regional Director, Great Lakes Federal
HTC Network

IN THIS ISSUE

04 BOARD
UPDATES

05 LEGISLATIVE
ADVOCACY DAY

06 NATIONAL WOMEN'S
CONFERENCE

11 MEDICARE
WEBINAR

13 PROTECT YOUR
MEDICAID COVERAGE

14 GOLF
CLINIC

18 FINANCIAL
ASSISTANCE

19 COMMUNITY NIGHT:
DECORATE A LUNCH BOX

20 LAUNCH PROGRAM &
NATIONAL EXCELLENCE

22 UNITE
WALKS

24 COMMUNITY NIGHT:
INCREDIBLE MO'S

25 YEAR END
APPEAL

26 CAMP OLD BEAGLE
ALUMNI CAMP

27 RARE
AND AWARE

THANK YOU
TO OUR
CONTRIBUTING
AUTHORS

HFM BOARD OF DIRECTORS

Troy Briggs, Treasurer
Kathleen Donohoe
Kathy Fessler, MD, PhD
David Hart
Ismael Jaber
Elliot Jones, Vice President
Jim Mohnach, President
Patrick Monks, Secretary
Jason Rogers

EX OFFICIO

Susan Fenters Lerch

HFM STAFF

Sharon Ceci*, Finance and Operations
Director
Lisa Clothier*, LMSW, ACSW, LCSW,
Outreach and Community Education
Manager
Meghan Mair Crosslin, Marketing and
Communications Director
Gwyn Hulswit, MA, Associate Director
David L. Kaumeyer*, EdD, Finance and
Operations Manager
Susan Fenters Lerch, Executive Director
Shari Luckey, MA, Program Services
Manager
Carrie McCulloch, Development Director
Laura Olson*, Office Manager
Sarah Procaro, Advocacy Director
Alyssa Silletti, MSW, Program Services
Manager
Anthony Stevens, Associate Camp
Director and Educational Services
Manager
Patrice Thomas, MS, MSW, Program
Services Director
Tim Wicks, Camp and Youth Services
Director

CONTRACTORS

Ashley Fritsch*, MS, Unite Walk
Sarah Spencer*, RN, BSN, MPA, Eagle
Journeys Camp Programs
*Additional Camp and Program contractors**

GREAT LAKES - V-E FEDERAL HTC NETWORK REGIONAL CORE CENTER

Sue Adkins*, BSN, RN, Regional
Program Manager
Lisa Clothier*, LMSW, ACSW, LCSW,
Regional Program Manager
Susan Fenters Lerch, Regional Director
Travis Tussing, JD, Regional
Administrator, Federal Grants Director
Amy Shapiro*, MD, Medical Director,
Chair, Regional Advisory Council

**Denotes part-time*

PAID CONSUMER OUTREACH
INCLUDED IN THIS NEWSLETTER

HFM IS HAPPY TO WELCOME THREE NEW MEMBERS TO HFM'S BOARD OF DIRECTORS.



David Hart, Director
Michigan Money Management, LLC,
Vice President of Wealth Management



Ismael Jaber, Youth Representative Director
Michigan State University, Student



Jason Rogers, Director
Michigan Veteran Affairs Agency,
Data and Budget Specialist

WE ARE INCREDIBLY GRATEFUL TO OUR RETIRING BOARD MEMBERS FOR THEIR DEDICATION AND SERVICE TO THE MICHIGAN BLEEDING DISORDERS COMMUNITY. THEY RETAINED THEIR SEATS ON THE BOARD THROUGHOUT THE UNPRECEDENTED CHALLENGES OF THE COVID-19 PANDEMIC, SUPPORTING THE FOUNDATION IN CONTINUOUSLY PROVIDING PROGRAMS AND SERVICES TO ALL AFFECTED BY BLEEDING DISORDERS.



Matt Sterling



Ronia Cole



Jeeva Nadarajah



Patients from across the state, and representatives from leading patient advocacy organizations, traveled to Lansing to share their personal stories with lawmakers and ask the Senate to hold a hearing on House Bill 4353, legislation that will guarantee insurance companies count all copayments made by or on behalf of Michiganders.

HB 4353, championed by House Health Policy Committee Chair Bronna Kahle, will reform an increasingly common insurance practice called copay accumulator adjustment programs that exclude assistance payments from counting towards a patient's deductible or out-of-pocket costs. Patients with bleeding disorders, and with other common chronic conditions like diabetes, arthritis, and cancer, rely on copay assistance programs to help them afford the medications they need to manage their conditions. Without assistance that counts, patients are forced to pay thousands of dollars out of pocket or go without their lifesaving treatments.

The legislation was approved nearly unanimously by the House in March 2021. Unfortunately, patients' access to assistance that counts has been stalled in the state Senate for 18 months.

MI ALL COPAYS COUNT LEGISLATIVE ADVOCACY DAY

HFM WAS PLEASED TO HOST THE MI ALL COPAYS COUNT (MACC) COALITION LEGISLATIVE ADVOCACY DAY ON WEDNESDAY, SEPTEMBER 28, 2022.



Representative Bronna Kahle, sponsor of HB 4353, and Representative Yousef Rabhi, spoke to patient advocates during the MACC Legislative Day. We are grateful for their support.

Patients and advocates met with their house Representatives to thank them for voting YES on HB 4353, and with their state Senators to ask them to support the bill and hold a hearing as soon as possible.

Thank you to all the advocates who joined us in Lansing to share the patient experience and the urgency for action. If you were unable to attend, you can still support the efforts by going to mirxcoalition.com and sending an email to your Senator! 💚

THANK YOU TO OUR SPONSOR

abbvie

HFM does not endorse any specific product or company.



NATIONAL CONFERENCE FOR WOMEN AND TEENS WITH HEMOPHILIA AND RARE FACTOR DEFICIENCIES

The themes for the 2022 conference centered on storytelling, and women and girls shared community challenges. Women's stories carry the legacy of being born into a family with an inheritable bleeding disorder, or a family with a spontaneously developed bleeding diagnosis, and all the joys and pain this experience contains. Bringing women together for this weekend provided the opportunity for community connections and mutual support.

EDUCATIONAL SESSIONS AND PROGRAMMING

Friday evening's keynote speaker, Stephanie Sarazin, spoke to attendees about her new book *Soulbroken: A Guidebook for Your Journey Through Ambiguous Grief*. With her quick wit and warm demeanor, Stephanie told her challenging story of family changes and the steps she took to recover and arrive at a place of reconciliation. Ambiguous grief refers to life events that take place on a different level than the death of a loved one. Whether it's a divorce, the development of a chronic health condition, or other significant losses that do not include death, all of us have experienced ambiguous grief. Stephanie spoke about the hope of working through these challenges, and how helpful it is to find "your people," others that can share life's challenges together. HFM provided a copy of *Soulbroken* to each attendee.

Our panel on Saturday morning addressed legal changes to US reproductive health laws and the significant challenges they present for women with bleeding disorders. All women need access to care, especially if they experience an emergency when pregnant. A miscarriage may initiate a severe bleeding episode which needs to be addressed by performing a medical procedure that, in some states might be illegal, putting the woman in a life-threatening situation. To address this complicated issue, the panel brought together four speakers and a moderator to explore this critical issue for women and girls with bleeding disorders.

WE WERE JOINED BY 188 WOMEN AND TEENS!

- 135 WITH HEMOPHILIA A
- 27 WITH HEMOPHILIA B
- 1 CARRIER OF HEMOPHILIA A
- 27 RARE FACTOR DEFICIENCIES
- 2 DUAL DIAGNOSES



HFM WAS EXCITED TO PLAN AND FACILITATE THE 5TH ANNUAL NATIONAL CONFERENCE FOR WOMEN AND TEENS WITH HEMOPHILIA AND RARE FACTOR DEFICIENCIES. AFTER TWO YEARS OF HOSTING A VIRTUAL CONFERENCE, IT WAS WONDERFUL TO COORDINATE A HYBRID EXPERIENCE FOR 188 WOMEN AND TEENS DURING THE WEEKEND OF OCTOBER 7-9, 2022.

The panel was moderated by Ann-Marie Nazzaro, PhD, co-founder of the Foundation for Women and Girls with Blood Disorders; speakers included:

- Miriam Goldstein, JD, Policy Director and Principal Legal Counsel, Hemophilia Federation of America, Washington, DC
- Magdalena Lewandowska, MD, Adult Hematologist, Indiana Hemophilia and Thrombosis Center, Indianapolis, IN
- Shaveta Malik, MD, Obstetrics and Gynecology, Buffalo, NY
- Keri Norris, PhD, MPH, MCHES, Vice President, Health Equity, Diversity, & Inclusion, National Hemophilia Foundation, Atlanta, GA

Ann-Marie quoted Dr. Audra H. James in her opening remarks, "Birth is a bleeding experience, and as women with bleeding disorders, we need to be informed about the laws that impact our bodies and vote for legislators that best represent our interests." Keri Norris spoke about access to care and health equity. Bleeding disorder patients should seek the implementation of patient centered initiatives, which supports the comprehensive care model, taking into consideration all aspects of a patient's life. Drs. Lewandowska and Malik spoke to the history of hemophilia

care and how long it took for women with hemophilia to be seen as patients with hemophilia, their treatment needs for hemophilia-related symptoms, and to not be seen as "just carriers." Dr. Malik spoke specifically about abortion and related procedures that can be life saving for a woman experiencing a miscarriage or ectopic pregnancy. This is critical information for women to have so they can advocate for their medical needs. Miriam Goldstein concluded the panel by viewing these challenges through an advocacy lens, and alerted attendees to the possibility of legislation being passed that would restrict women and girls' access to contraception such as IUDs and hormonal treatment for heavy bleeding. She encouraged women who encounter pharmacists who refuse to fill a prescription for one of these items to visit Hemophilia Federation of America's website for more resources, and to add their story to HFA's compilation of stories used for policy advocacy.

In addition to speakers and panels, this year's conference offered thirteen breakout sessions on topics ranging from Debunking the Myth of Wonder Woman to an update on rare factor deficiencies. A session was offered to women who wanted to continue to explore grief issues. Another session was offered to talk about the impact of the HIV crisis on individuals, families, and the community.

ATTENDEES JOINED US FROM THE FOLLOWING STATES/AREAS:

AR, AZ, CA, CO, DE, FL, GA, IA, ID, IL, IN, KS, LA, MA, MD, ME, MI, MO, MS, NC, NE, NH, NM, NV, NY, OH, OK, OR, PA, SC, TN, TX, VA, WA, WI, PUERTO RICO, AND NEW ZEALAND.

Present during the weekend were two emotional support social workers from our Michigan HTC's: Karen Boyd, LMSW, Michigan State University, Center for Bleeding and Clotting Disorders and Dave Rushlow, LMSW, Northern Michigan Bleeding Disorders Center at Munson Healthcare. We greatly appreciate their clinical services in helping individual attendees process their experiences while participating in the conference. We also greatly appreciate the medical support from Kathi Sheldon, RNC Nurse Coordinator, Northern Michigan Bleeding Disorders Center at Munson Healthcare, who assisted with Covid testing and medical challenges that arose over the weekend.

YOUNG ADULT PROGRAMMING

Teens and young adults who joined us in-person had the opportunity to participate in sessions especially for them! A Q&A session on reproductive health created an open dialogue for young adults to ask questions and hear from other individuals their age with similar shared experiences. The relationships continued to grow as the Corner Health Center of Ypsilanti, MI joined us for an afternoon of informative and engaging activities. The Theatre Troupe performed a short skit on how depression and anxiety can impact relationships and the Youth Leadership Council guided us through a workshop on leadership styles. We then practiced communicating with different leadership styles by working in teams to build the tallest marshmallow-spaghetti tower. It was an afternoon full of curiosity, laughter, and new friendships!

INTERGENERATIONAL STORYTELLING WORKSHOP

One sentence is all it took for many women in the room to be moved to tears. By the end of Saturday evening's storytelling workshop, each attendee who chose to participate in the workshop had shared with the group one sentence that encapsulated their journey. Pain, grief,

and resiliency were common themes amongst the stories, many of which were spoken out loud for the first time. The vulnerability of the women's stories was a testament to workshop facilitators, Adela Nieves Martinez and Diana Seales, who created a warm and intimate space for women to connect. Adela and Diana opened the workshop with a land acknowledgement recognizing that we're currently residing on the traditional land and territory of the Anishinaabe people, and a smudge to cleanse the space and ground folks in the present moment.

Women had brought items from home that represented their journey and shared their meanings in small groups. We were then guided through the process of developing and telling our stories and given the opportunity to free write. While some women were eager to record their stories that evening, others desired additional time to process and construct their story. Their voices will contribute to a growing library of stories highlighting the experiences of women in the bleeding disorders community.

The weekend offered many educational sessions and opportunities to connect, learn, and engage with others who share similar experiences and challenges. We look forward to utilizing the feedback about this year's conference to continue to build and address the needs of women and teens with hemophilia and rare factor deficiencies. Thanks to all HFM staff, women and teen attendees, our speakers, and our sponsors for creating a memorable experience! 💜

THANK YOU TO OUR SPONSORS

REGIONAL SUPPORTER



COMMUNITY LEAD SPONSORS



COMMUNITY SPONSOR



COMMUNITY SUPPORTERS







sanofi

Connected. Together.

For me, being a Community Relations and Education (CoRe) Manager for Sanofi is about empowering the hemophilia community by providing a personal connection to education and resources.

Rebecca Gorde
CoRe Manager for
Michigan & Indiana

Stay connected.

248-915-5282

rebecca.gorde@sanofi.com

RareBloodDisorders.com

[!\[\]\(17413706fd4997a1a4bdf85c6864eee1_img.jpg\)](#) [!\[\]\(f419710cbe076aa30a9c6c031b5cbe84_img.jpg\)](#) [!\[\]\(2726020a4107bdc9042b257034f90eb3_img.jpg\)](#) @HemophiliaCoRes




2022 Genzyme Corporation. All rights reserved.
Sanofi is a registered trademark of Sanofi or an affiliate.
MAT-US-2100171-v2.0-04/2022

PAID CONSUMER OUTREACH

MEDICARE WEBINAR

HFM was pleased to partner with the Area Agency on Aging's Medicaid Medicare Assistance Program (MMAP) to provide a thorough overview of Medicare. As we know in the bleeding disorders community, insurance is a lifeline for patients with chronic conditions who rely on consistent care and treatment; unfortunately, it can be extremely complex to navigate.

Our guest speaker from MMAP, Melissa Devine, joined us to help make this large and intricate topic more manageable for members of the community. She started by explaining what Medicare is and the different parts and plans available. We learned the process for enrolling and the importance of paying attention to enrollment deadlines. Most importantly, we learned that each of us needs to choose the best plan for us based on our individual health needs.

If you need assistance with Medicare, please reach out to the Area Agency on Aging's Medicare Medicaid Assistance Program. MMAP can help you solve problems, find answers, and understand your options. This assistance is unbiased and free of cost. 

Learn more at www.aaa1b.org/medicare-medicaid-help

THANK YOU TO OUR SPONSORS



CSL Behring

Genentech
A Member of the Roche Group

HFM does not endorse any specific product or company.



OUR VISION: A WORLD WHERE NO LIFE IS LIMITED BY GENETIC DISEASE



Learn about gene therapy
research for hemophilia.



Interested in enrolling in a
Spark-sponsored hemophilia
gene therapy clinical trial?



Want to know more about
gene therapy clinical trials?

**At Spark® Therapeutics, we are committed to
discovering, developing and delivering gene therapies.**

**Discover more about
gene therapy research**



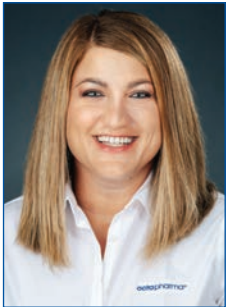
© 2022 Spark Therapeutics, Inc. N-HEM-US-420121-3
Spark, Spark Therapeutics and its design, and Hemophilia Forward and its design, are trademarks, registered marks, or service marks of Spark Therapeutics, Inc. in the United States.
1-855-SPARKTX

wilate®

von Willebrand
Factor/Coagulation
Factor VIII Complex
(Human)

www.wilateusa.com

Contact your **Octapharma Representative:**



Bri Vieke

PHONE | 551.502.7007

EMAIL | briana.vieke@octapharma.com



Resources for patients and caregivers, including free trial and co-pay assistance, educational materials, and community connection.

**Join the program at www.factormyway.com
or call 1-855-498-4260.**

PROTECT YOUR MEDICAID COVERAGE

In the beginning of the COVID pandemic, the federal government enacted a federal Public Health Emergency (PHE) that included changes to Medicaid eligibility, administration, and policies to help prevent Medicaid beneficiaries from losing their health care coverage during the pandemic. Specifically, the PHE included a continuous coverage rule that said people on Medicaid could not be disenrolled unless they moved to another state, died, or asked to be removed.

It is anticipated that the US Department of Health and Human Services will soon announce the end of the federal PHE. Once the PHE ends, Michigan will restart Medicaid eligibility renewals.

Renewal packets will be mailed to the addresses they have on file. Packets are anticipated to go out one to two months before the month they are due.

YOU MUST RESPOND TO THIS RENEWAL IF YOU RECEIVE IT, OTHERWISE, YOUR MEDICAID COVERAGE WILL STOP

TIMELINE AND PROCESS

To simplify the complexity of the PHE unwinding process, the Michigan Department of Health and Human Services (MDHHS) will maintain beneficiaries' current renewal month in their case records and conduct a full redetermination at the next scheduled renewal month following the end of the PHE. The entire redetermination process is anticipated to take a year, so even if the PHE ends, you may not receive your renewal documents right away.

While MDHHS has indicated that continuity of coverage is a priority for them, it is anticipated that many eligible individuals will lose coverage due to administrative errors or missed communication. 💧

PROTECT YOUR HEALTH CARE COVERAGE

- Update your contact information with the Michigan Department of Health and Human Services (MDHHS)
- Visit [Michigan.gov/MIBridges](https://www.michigan.gov/MIBridges) and make sure your address, phone number, and email address are up to date
- You can also call your local MDHHS office for help. Local office information can be found at www.michigan.gov/mdhhs/inside-mdhhs/county-offices
- Check your mail
- If you get a renewal packet, be sure to fill it out, sign the forms, and send them back by the due date

INSPIRING SHOTS

KELLY HWANG, COMMUNITY MEMBER

CSL Behring

Over the summer there were four weekends where members and caregivers of the Michigan bleeding disorders community were invited to participate in the Inspiring Shots Golf Clinic through the Wingmen Foundation. These clinics were supported by

CSL Behring, who also brought in professional golfer Perry Parker to host the first session. I was lucky enough to attend three of these clinics and I can certainly say that I'm very thankful that I did. During these clinics, I got to connect with community members that I hadn't seen in a long time as well as meet new friends who I now get to share another common interest with – golf! These clinics were coordinated and run by amazing golf pros who really took the time to teach the fundamentals to newcomers of the sport as well as refine the existing skill of active players. Over the course of the four sessions, we learned positioning, the differences between clubs, and the basics of the golf game. We were able to adjust our

swings for using drivers, irons, woods, and our putters. With these adjustments and new knowledge, improvements and smiles were seen all around! Time was spent on both the driving range and a regular course to allow for realistic play.

This experience allowed me to renew my love for the sport that I had taken a few years off from, improve my skill, and make new friends...I would say that's pretty inspiring. When the clinic rolls around again next year, I certainly plan on attending again as well as encouraging others to attend as well, with or without experience. Thank you again for bringing this valuable clinic to the community and furthering connections both on and off the course. 📌



HFM does not endorse any specific product or company.

Biotherapies for Life® **CSL Behring**

Proud to serve the bleeding disorders community with the latest technologies, innovative therapies and support.

For additional information please contact:
Lauren Hecht MA, LLP
Senior Manager Coagulation Products
248-310-0202
Lauren.Hecht@CSLBehring.com





Connected to you.

As Community Relations & Education Managers, our work with the hemophilia community is deeply personal. It unites us in our efforts to help educate and support you and your family.



Reach out to your local CoRe to learn more.
rareblooddisorders.com

   @HemophiliaCoRes | 1-855-SGZHEME

PAID CONSUMER OUTREACH

SANOFI GENZYME 

© 2022 Genzyme Corporation. All rights reserved. MAT-US-2101737-v1.0-07/2022

NOW APPROVED FOR ONCE-WEEKLY PROPHYLAXIS

BE READY FOR THE UNEXPECTED

ONCE-WEEKLY REBINYN® HELPS YOU KEEP FACTOR 9 LEVELS HIGHER FOR LONGER^a

Timothy has severe hemophilia B and uses Rebinyn®.

Expect higher factor levels and bleed protection with once-weekly Rebinyn®.^b



With once-weekly Rebinyn®, adults and adolescents with hemophilia B can spend approximately 80% of their week with Factor 9 levels in the non-hemophilia range (greater than 40%).^b



in adults treated with Rebinyn® 40 IU/kg^c



in adolescents treated with Rebinyn® 40 IU/kg^c

Visit www.rebinyn.com to learn more about once-weekly Rebinyn®

^aRebinyn® achieved and maintained higher factor levels than recombinant Factor 9 based upon a phase 1 study comparing 25, 50, and 100 IU/kg doses of Rebinyn® to a 50 IU/kg dose of standard half-life recombinant Factor 9 in 7 adults and a 50 IU/kg dose of plasma-derived Factor 9 in 8 adults. For Rebinyn®, estimated average Factor 9 activity is adjusted to a dose of 50 IU/kg. Incremental recovery at 30 minutes (IR₃₀) and half-life were higher and longer with Rebinyn® than recombinant Factor 9 (IR₃₀ 0.0131 vs 0.0068 (IU/mL)/(IU/kg) and half-life 93 vs 19 hours). The clinical relevance of these pharmacokinetic differences is unknown.

Incremental Recovery: The increase in plasma concentration per IU/kg of factor administered.

Half-life: The time it takes for the level of factor in the blood to fall by half (50%).

^bData represent mean steady-state pharmacokinetic (PK) profiles from previously treated adolescent/adult patients with moderate-to-severe hemophilia B (N=9) taking repeated doses of Rebinyn® 40 IU/kg once weekly. Factor 9 levels were within the non-hemophilia range (greater than 40%) for 5.4 days (about 80% of the week).

^cBased on analysis using a 1-stage assay in patients (N=6) aged 18 and older, the half-life at steady state was 115 hours following once-weekly (40 IU/kg) dosing; in patients (N=3) aged 13 to 17, the half-life at steady state was 103 hours. Following single-dose administration (40 IU/kg) in the same patient population, the half-life was 83 hours (adults) and 89 hours (adolescents).

Indications and Usage

What is Rebinyn® Coagulation Factor IX (Recombinant), GlycoPEGylated?

Rebinyn® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Rebinyn® is used to treat, prevent, or reduce the frequency (number) of bleeding episodes in people with hemophilia B. Your healthcare provider may give you Rebinyn® when you have surgery. Rebinyn® is not used for immune tolerance therapy.

Important Safety Information

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

- Do not attempt to do an infusion yourself unless you have been taught how by your healthcare provider or hemophilia treatment center. Carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing Rebinyn®.

Who should not use Rebinyn®?

Do not use Rebinyn® if you:

- are allergic to Factor IX or any of the other ingredients of Rebinyn®.
- are allergic to hamster proteins.

What should I tell my healthcare provider before using Rebinyn®?

Tell your healthcare provider if you:

- have or have had any medical conditions.
- take any medicines, including non-prescription medicines and dietary supplements.
- are nursing, pregnant, or plan to become pregnant.
- have been told you have inhibitors to Factor IX.

How should I use Rebinyn®?

- Rebinyn® is given as an infusion into the vein.
- Call your healthcare provider right away if your bleeding does not stop after taking Rebinyn®.
- Do not stop using Rebinyn® without consulting your healthcare provider.

What are the possible side effects of Rebinyn®?

- Common side effects include infusion site reaction (bruising, bleeding, swelling, pain, or redness), itching, and rash.
- Your body can also make antibodies called "inhibitors" against Factor IX, including Rebinyn®, which may stop Rebinyn® from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.
- Call your healthcare provider right away or get emergency treatment right away if you get, for example, any of the following signs of an allergic reaction: hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.
- You may be at an increased risk of forming blood clots in your body, especially if you have risk factors for developing blood clots. Call your healthcare provider if you have chest pain, difficulty breathing, leg tenderness, or swelling.
- Animals given repeat doses of Rebinyn® showed Polyethylene Glycol (PEG) in certain cells in the brain. The potential human implications of these animal tests are unknown.

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

Rebinyn® is a prescription medication.

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.



Novo Nordisk Inc., 800 Scudders Mill Road, Plainsboro, New Jersey 08536 U.S.A.

Rebinyn® is a registered trademark of Novo Nordisk Health Care AG.

Novo Nordisk is a registered trademark of Novo Nordisk A/S.

© 2022 Novo Nordisk Printed in the U.S.A. US22REB00167 October 2022

PAID CONSUMER OUTREACH

rebinyn®
Coagulation Factor IX
(Recombinant), GlycoPEGylated

rebinyn®**Coagulation Factor IX
(Recombinant), GlycoPEGylated****Brief Summary Information about:****REBINYN® Coagulation Factor IX
(Recombinant), GlycoPEGylated****Rx Only**

This information is not comprehensive.

- Talk to your healthcare provider or pharmacist
- Visit www.novo-pi.com/REBINYN.pdf to obtain FDA-approved product labeling
- Call 1-844-REB-INYN

Read the Patient Product Information and the Instructions For Use that come with REBINYN® before you start taking this medicine and each time you get a refill, as there may be new information.

This Patient Product Information does not take the place of talking with your healthcare provider about your medical condition or treatment. If you have questions about REBINYN® after reading this information, ask your healthcare provider.

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

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

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

What is REBINYN®?

REBINYN® is an injectable medicine used to replace clotting Factor IX that is missing in patients with hemophilia B. Hemophilia B is an inherited bleeding disorder in all age groups that prevents blood from clotting normally.

REBINYN® is used to treat, prevent, or reduce the frequency (number) of bleeding episodes in people with hemophilia B.

Your healthcare provider may give you REBINYN® when you have surgery.

Who should not use REBINYN®?

You should not use REBINYN® if you

- are allergic to Factor IX or any of the other ingredients of REBINYN®
- if you are allergic to hamster proteins

If you are not sure, talk to your healthcare provider before using this medicine.

Tell your healthcare provider if you are pregnant or nursing because REBINYN® might not be right for you.

What should I tell my healthcare provider before I use REBINYN®?

You should tell your healthcare provider if you

- Have or have had any medical conditions.
- Take any medicines, including non-prescription medicines and dietary supplements.
- Are nursing. It is not known if REBINYN® passes into breast milk or if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if REBINYN® may harm your unborn baby.
- Have been told that you have inhibitors to Factor IX (because REBINYN® may not work for you).

How should I use REBINYN®?

Treatment with REBINYN® should be started by a healthcare provider who is experienced in the care of patients with hemophilia B.

REBINYN® is given as an infusion into the vein.

You may infuse REBINYN® 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 hemophilia treatment center or healthcare provider. Many people with hemophilia B learn to infuse the medicine by themselves or with the help of a family member.

Your healthcare provider will tell you how much REBINYN® to use based on your weight, the severity of your hemophilia B, and where you are bleeding. Your dose will be calculated in international units, IU.

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

If your bleeding is not adequately controlled, it could be due to the development of Factor IX inhibitors. This should be checked by your healthcare provider. You might need a higher dose of REBINYN® or even a different product to control bleeding. Do not increase the total dose of REBINYN® to control your bleeding without consulting your healthcare provider.

Use in children

REBINYN® can be used in children. Your healthcare provider will decide the dose of REBINYN® you will receive.

If you forget to use REBINYN®

If you forget a dose, infuse the missed dose when you discover the mistake. Do not infuse a double dose to make up for a forgotten dose. Proceed with the next infusions as scheduled and speak to your healthcare provider if you have any questions or concerns.

If you stop using REBINYN®

Do not stop using REBINYN® without consulting your healthcare provider.

If you have any further questions on the use of this product, ask your healthcare provider.

What if I take too much REBINYN®?

Always take REBINYN® exactly as your healthcare provider has told you. You should check with your healthcare provider if you are not sure. If you infuse more REBINYN® than recommended, tell your healthcare provider as soon as possible.

What are the possible side effects of REBINYN®?

Common Side Effects Include:

- infusion site reaction (bruising, bleeding, swelling, pain, or redness)
- itching
- rash

Your body can also make antibodies called "inhibitors" against Factor IX, including REBINYN®, which may stop REBINYN® from working properly. Your healthcare provider may need to test your blood for inhibitors from time to time.

You could have an allergic reaction to coagulation Factor IX products. **Call your healthcare provider right away or get emergency treatment right away if you get, for example, any of the following signs of an allergic reaction:** hives, chest tightness, wheezing, difficulty breathing, and/or swelling of the face.

You may be at an increased risk of forming blood clots in your body, especially if you have risk factors for developing blood clots. Call your healthcare provider if you have chest pain, difficulty breathing, leg tenderness or swelling.

These are not all of the possible side effects from REBINYN®. Ask your healthcare provider for more information. You are encouraged to report side effects to FDA at 1-800-FDA-1088.

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

What are the REBINYN® dosage strengths?

REBINYN® comes in four different dosage strengths. The actual number of international units (IU) of Factor IX in the vial will be imprinted on the label and on the box. The four different strengths are as follows:

Cap Color Indicator	Nominal Strength
Red	500 IU per vial
Green	1000 IU per vial
Yellow	2000 IU per vial
Dark Gray	3000 IU per vial

Always check the actual dosage strength printed on the label to make sure you are using the strength prescribed by your healthcare provider.

How should I store REBINYN®?

Prior to Reconstitution (mixing the dry powder in the vial with the diluent):

Store in original package in order to protect from light. Do not freeze REBINYN®.

REBINYN® vials can be stored in the refrigerator (36-46°F [2°C-8°C]) for up to 24 months until the expiration date, or at room temperature (up to 86°F [30°C]) for a single period not more than 6 months.

If you choose to store REBINYN® at room temperature:

- Note the date that the product is removed from refrigeration on the box.
- The total time of storage at room temperature should not be more than 6 months. Do not return the product to the refrigerator.
- Do not use after 6 months from this date or the expiration date listed on the vial, whichever is earlier.

Do not use this medicine after the expiration date which is on the outer carton and the vial. The expiration date refers to the last day of that month.

After Reconstitution:

The reconstituted (the final product once the powder is mixed with the diluent) REBINYN® should appear clear without visible particles.

The reconstituted REBINYN® should be used immediately.

If you cannot use the reconstituted REBINYN® immediately, it should be used within 4 hours when stored at or below 86°F (30°C). Store the reconstituted product in the vial.

Keep this medicine out of the sight and out of reach of children.

What else should I know about REBINYN® and hemophilia B?

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

For more information about REBINYN®, please call Novo Nordisk at 1-844-REB-INYN.

Revised: 08/2022

REBINYN® is a trademark of Novo Nordisk A/S.

For Patent Information, refer to: <http://novonordisk-us.com/products/product-patents.html>

Manufactured by:
Novo Nordisk A/S
Novo Allé, DK-2880 Bagsværd, Denmark

More detailed information is available upon request.

Available by prescription only.

For information about REBINYN® contact:
Novo Nordisk Inc.
800 Scudders Mill Road
Plainsboro, NJ 08536, USA

© 2022 Novo Nordisk
US22REB00153 August 2022



FINANCIAL ASSISTANCE: BECAUSE ENSURING THE WELL-BEING OF OUR COMMUNITY IS MORE THAN MEDICINE

DID YOU KNOW THAT ALONG WITH PARTNER ORGANIZATIONS, HFM HAS PROVIDED FINANCIAL ASSISTANCE TO:

236 COMMUNITY MEMBERS*

If not, this may be due in part to the fact that HFM values the privacy of recipients of this program and keeps the assistance completely confidential. We are gratified to assist with emergent needs such as housing evictions, utility shutoffs, and other situations.

Why is this program so important? Health and safety go hand in hand with housing and food security.

Utilities can be the key to functioning medical equipment, the ability to store food, warmth in the winter, and a positive environment for children to live in. It is our bleeding disorders that bring us together, but we recognize that it is the whole person, each in their own unique situation that we serve, even when times are tough. ♡

More details about this program can be found at: www.hfmich.org/get-help

WE WOULD NOT BE ABLE TO PROVIDE THIS PROGRAM WITHOUT THE AMAZING HELP OF



**Numbers reported from January through October 2022*

WORKING TOGETHER: THE HEMOPHILIA FEDERATION OF AMERICA'S ASSISTANCE PROGRAMS

CYNTHIA CROSS, SERVICES MANAGER, HEMOPHILIA FEDERATION OF AMERICA

In 2022 the Hemophilia Federation of America (HFA) provided financial assistance to 447 community members and their families throughout the United States. HFM has referred community members to and shared information about HFA's programs, which has enabled HFA to provide direct financial assistance to 26 families in Michigan through HFA's Helping Hands programs. HFA's Helping Hands programs provide emergency financial assistance for household bills, medically necessary

items not covered by insurance, financial assistance when recovering from a natural disaster, and medical travel and educational supplies for community members with inhibitors.

HFA's Helping Hands team acknowledges some community members need additional support, so the team maintains an up to date and robust list of resources in their Patient Assistance Portal for financial assistance, mental health, drug manufacturer programs, financial

planning, and employment rights/search. In addition to Helping Hands, HFA offers financial education resources, webinars, and a Job Readiness Grant through their Helping Forward program. Without organizations such as HFM referring community members and sharing information about HFA's programs, HFA would not have the ability to connect with community members in need and offer support to those experiencing hardship. ♡

COMMUNITY NIGHT: DECORATE A LUNCHBOX



We may have been in different physical locations, yet the Michigan bleeding disorders community brought loads of energy to our virtual community night in August. Families from across the state of Michigan joined us to celebrate the beginning of a new school year with an evening of laughter and creativity. Attendees were given a variety of craft supplies to decorate a HFM lunchbox, many of which were transformed into scary and fun lunchbox monsters!

HFM does not endorse any specific product or company.

To learn more about making healthy choices at mealtime, we played an interactive online game where we traveled to different countries to learn about meals eaten around the world. We worked as a team to choose our daily meals based on their nutritional value and budget. The group indulged on atassi in Benin, num banh chok in Cambodia, and apple pie a la mode in the United States for dessert. It was a fun-filled evening for the entire family! 🍷

THANK YOU TO OUR SPONSORS

SOARING EAGLE SPONSOR

sanofi

HAPPY CAMPER SPONSOR



FRIENDS OF HFM

octapharma

EVENT SPONSOR



Jivi
antithrombotic factor
(recombinant) PEGylated-auct

Jivi®, now with up to 7 YEARS OF DATA.

Talk to your doctor about the study.



Scan this QR code to learn more about
the data at JiviExtensionStudy.com



©2022 Bayer. All rights reserved. Bayer, the Bayer Cross, and Jivi are registered trademarks of Bayer. 6/22 PP-JIV-US-1787-1 PAID CONSUMER OUTREACH

LAUNCH

THANK YOU TO OUR SPONSORS



Genentech
A Member of the Roche Group

HFM's LAUNCH program continues its purpose in providing certifications, job readiness, and training to those in the bleeding disorders community. While there has been much conversation about employment opportunities and wage growth in the last year, many individuals in the bleeding disorders community continue to experience difficulty finding job placements that accommodate their needs. LAUNCH provides individual support and assistance for members of the community who are seeking further education or attainment of certification to assist in the employment process. We are proud of all of the accomplishments of our graduates this year. 💧

HFM does not endorse any specific product or company.



HFM AWARDED FOR NATIONAL EXCELLENCE

HFM was recognized this year by the National Hemophilia Foundation (NHF) for national excellence in three different areas. In the late spring, NHF selected HFM as the chapter with the most engaging social media campaign during Bleeding Disorders Awareness Month in March. Just in time for NHF's Bleeding Disorders Conference in August, HFM was additionally recognized as a leader in our camp program and in our advocacy work. Susan Fenters Lerch, along with other HFM staff and Michigan community members, accepted the Val Bias and Todd Smith Innovation in Camp award as well as the John Indence award, a special award that recognizes a chapter's exceptional work elevating advocacy issues, and raising awareness and funds during Bleeding Disorders Awareness Month through World Hemophilia Day and the state-based advocacy season.

We are thrilled with these moments of recognition and share this excitement with the entire Michigan community with a special thank you to all of you who supported and participated in these efforts. Congratulations! 💧





1970

*First patients
ever receive
gene therapy*

1997

*First rFIX
products
approved
by FDA*

1999

*First gene
therapy
trial in hem B*

2018

*Late-stage
trials for
gene therapy
in hem B
underway*

EVERY STEP HAS BEEN EVOLVING THE SCIENCE OF GENE THERAPY IN HEMOPHILIA B

Explore the advancing science behind
gene therapy at [HemEvolution.com](https://www.HemEvolution.com)

**We're working to make gene therapy a reality
for you and your patients with hemophilia B.**



Biotherapies for Life® is a registered trademark of CSL Behring LLC.
1020 First Avenue, PO Box 61501, King of Prussia, PA
©2022 CSL Behring LLC www.CSLBehring.com

Biotherapies for Life® **CSL Behring**

Unite

for Bleeding Disorders

MICHIGAN WALKS

GRAND RAPIDS

DONUTS! A KICKOFF TO FALL! AND...RAIN!

This year, the Unite for Bleeding Disorders West MI walk had what was, quite frankly, an awesome location. Robinette's Apple Haus promised a beautiful walk through orchards, fresh doughnuts, and the opportunity to get into a fall mood just as school was starting. Mother Nature, on the other hand, promised to dash those hopes when she revealed a 90+% chance of pouring rain on our walk day. We had the tents, we had the spirit, we even had the donuts, but would our walkers show up?

Oh, West Michigan, we love you so. You brought your smiles, positive attitude, and willingness to go with the flow.

Our walk morphed into an HFM social hour under the tents. We improvised and you came along with us. Between our families, our team from Helen DeVos Children's Hospital HTC, and our HFM staff, this walk felt like a family reunion. We are so grateful for each of you.

Special thanks to Sarah Procaro for being willing to put her face on the line for our staff pie-in-the-face fundraiser challenge, and to Matt Sterling and Sarah Spencer who volunteered to walk the rain-drenched route to collect our route signs at the end. We were absolutely soaked with appreciation! 💧

DETROIT

A WHOLE NEW YEAR. A WHOLE NEW WALK!

2022 marked the return of the in-person Unite SE Michigan Walk since the beginning of the pandemic, and boy were we excited! The opportunity to see everyone was a true highlight, along with some of the new perks that came with our new walk venue.

Kensington Metropark was a gorgeous location that allowed us to give our walkers cost-free registration. We haven't been able to do this for an in-person walk previously, and it felt great to extend it to our walkers! Additionally, the new location allowed us to provide everyone with lunch – hot dogs for the win! We had a dedicated stage and walk area, new games and activities, and day passes to the park for all attendees. But most

importantly, we had all of you with us. The combination of seasoned and new teams made the walk feel so special.

Ashley Fritsch, our walk coordinator, even offered walkers the chance to donate and select a staff member to receive a pie to the face! Congratulations (???) to Sue Lerch for “winning” that honor!

Thank you to all of our walkers. Thank you also to Matt Sterling and Sarah Spencer for serving as our route medics. Thanks to Larry West for helping with setup and tear-down. And finally, a very special thank you to Ashley Fritsch, who after a brief hiatus, returned temporarily to HFM just to plan and make sure that the walk was a great experience for all. We appreciate you, Ashley! 💧



THANK YOU TO OUR SPONSORS

NATIONAL PRESENTING PARTNERS



NATIONAL COMMUNITY PARTNERS



NATIONAL PARTNERS



HAPPY CAMPER SPONSORS



FRIENDS OF HFM SPONSOR



HFM does not endorse any specific product or company.

COMMUNITY NIGHT: INCREDIBLE MO'S

We missed seeing our Traverse City friends, and our Community Night at Incredible Mo's provided everyone the perfect opportunity to come together again! How fun is it to reunite after a couple of years of not seeing each other to enjoy educational learning, pizza, and bowling! Thank you to everyone that came out to enjoy some time together, share some smiles, and to keep our tradition of fun Community Nights alive. 🍷

HFM does not endorse any specific product or company.

THANK YOU TO OUR SPONSORS

SOARING EAGLE SPONSOR

sanofi

HAPPY CAMPER SPONSORS

CSL Behring



FRIENDS OF HFM SPONSOR

octapharma

COMMUNITY NIGHT SPONSORS

Genentech

A Member of the Roche Group

BioMarin

Hema Biologics

Medexus

Pfizer



Exploring the science behind gene therapy research

Gene therapy research has the potential to bring an entirely new option to people with specific genetic conditions. Many gene therapies are in clinical trials to evaluate the possible risks and benefits for a range of conditions, including hemophilia. HemDifferently is here with gene therapy education, providing accurate information on the basics and beyond.

What questions do you have? Get them answered. Explore gene therapy at **HemDifferently.com**

No gene therapies for hemophilia have been approved for use or determined to be safe or effective.

BioMARIN

©2020 BioMarin Pharmaceutical Inc. All Rights Reserved. MMRCGTH0226 0420

PAID CONSUMER OUTREACH



FOLLOW US!



www.hfmich.org/facebook



[@hfmich](https://www.instagram.com/hfmich)



www.hfmich.org/camp



www.hfmich.org

YOUR GIFT PROVIDES HOPE & CONNECTIONS

If you are reading this issue of the Artery, it is likely because you are connected to HFM and the Michigan bleeding disorders community. Have you ever participated in an HFM program? If yes, you directly know how important it is to come together for education, support, and fun. Has someone you love ever benefitted from an HFM service? If so, perhaps you have heard stories of connections made and questions answered. If you are a donor, this

is where you should pause to pat yourself on the back, because you played an important role in making these amazing moments happen! Yes, YOU!

Your influence is felt in everything we do. When we couldn't meet in person, HFM created virtual opportunities to move our mission forward. Then, as we began our return to in-person, we maintained selected virtual programming in

response to those unable to meet in person. Now we will move forward with new and innovative ways to keep the HFM community connected, informed, and supported, all thanks to YOU, our donors!

Thank you for supporting our mission to improve the lives of Michigan's bleeding disorders community. You truly make a difference. 💧

IT IS NOT TOO LATE TO MAKE A DIFFERENCE THIS YEAR

New and returning donors can make their tax-deductible donation with the goal of providing cost-free medical alert id tags and supporting HFM's life-changing programs such as camp, SpringFest, Women's programming, diagnosis-specific events, much-needed advocacy, aging programs, job-training... the list goes on and on. YOU can do all of this with your support through a donation today! Donors giving \$100 or more will be listed in HFM's Spring 2023 newsletter, the Artery.

CAMP OLD BEAGLE ALUMNI CAMP

In early September camp alumni – campers and camp staff – came together at Camp Old Beagle for a beautiful weekend at Pioneer Trails. Held after HFM's summer camping programs have concluded, Camp Old Beagle is a time for anyone age 18+ who has been involved in HFM's camp programs to reconnect with the community.

For those who have been part of camp, we know that camp holds a special place in our hearts. However, as many of us become busy with school, jobs, and family responsibilities, it can be difficult to find time to reconnect. Camp Old



Beagle helps rekindle and strengthen friendships formed at camp – whether the last time someone attended camp was just a year ago or three decades ago. And as some community members start new relationships and families, Camp Old Beagle also serves to familiarize partners and young children with both the camp program and with bleeding disorders in general.

Camp family is a forever bond. If you have ever been to camp in any capacity, you are always welcome at Camp Old Beagle. 🍷

HISTORY/BASIC INFORMATION

Two patients were identified in the 1950s with a new bleeding disorder that would eventually be called factor X deficiency. Their surnames were Stuart and Prower, and FXD was initially referred to as Stuart-Prower factor deficiency. The factor X (FX) molecule was first identified in 1955. It is one of the vitamin K-dependent plasma proteins and converts prothrombin to thrombin which allows for fibrin clot formation.

SYMPTOMS

People with FXD can have various bleeding manifestations. Bleeding is often related to the baseline FX level of the individual and is more severe in people with circulating FX levels less than 10%. Umbilical cord stump bleeding is one of the first symptoms a newborn may have with this diagnosis. Other bleeding symptoms may include easy bruising, mouth/gum bleeding, nosebleeds, blood in the urine, gastrointestinal (GI) bleeding, joint and/or muscle bleeding, bleeding in the brain, and bleeding with trauma or surgery. Women with FXD can have heavy menstrual bleeding and bleeding after childbirth.

DIAGNOSIS

The diagnosis of FXD is made by taking a personal and family medical history, doing a physical exam, and performing laboratory testing. Factor X assay testing is done to confirm FXD diagnosis. The level will be lower than the normal range of roughly 50-150%. People can have severe (<10%), moderate (10-40%), or mild (40-50%) FXD.

Acquired forms of FXD exist often due to other conditions such as amyloidosis, some forms of cancer, liver disorders, vitamin K deficiency, and with some medications. Managing the underlying cause of acquired FXD helps reduce bleeding complications.

INHERITANCE

Both parents will need to pass on the gene to their child for symptoms to be present. If only one FX gene variant is present in an individual, the person is considered a carrier. The FX gene is located on chromosome 13. There are several gene mutations that can lead to FXD. It is estimated that FXD affects about one in every 500,000-1,000,000 people.

REFERENCES:

- Peyvandi f, et al. Blood Reviews. 2021; <https://doi.org/10.1016/j.blre.2021.100833>.
- Chatterjee T, et al. Med J Armed Forces India. 2015 Jul;71(Suppl 1):S184-6. doi: 10.1016/j.mjafi.2014.01.007. Epub 2014 Apr 3. PMID: 26265825; PMCID: PMC4529547.
- <https://rarediseases.org/rare-diseases/factor-x-deficiency>. Accessed October 10, 2022.
- <http://www.rarecoagulationdisorders.org/diseases/factor-x-deficiency/disease-overview>. Accessed October 10, 2022.
- <https://www.hemophilia.org/healthcare-professionals/allied-healthcare/nursing/nurses-guide-to-bleeding-disorders>. Rare Bleeding Disorder chapter. Accessed October 10, 2022.
- Pagana KD, et al. Mosby's Diagnostic and Laboratory Test Reference. 14th Ed. Elsevier; 2019.
- Kulkarni R, et al. J Thromb Haemost. 2018; 16:849-857.

RARE AND AWARE

JIM MUNN, MS, BSN, RN-BC, PROGRAM NURSE COORDINATOR,
UNIVERSITY OF MICHIGAN HEMOPHILIA TREATMENT CENTER

WELCOME TO THE SECOND EDITION OF DISCUSSIONS ON RARE BLEEDING DISORDERS (RBDS). THE GOAL OF THIS ARTICLE IS TO HIGHLIGHT A UNIQUE COAGULATION PLASMA PROTEIN DEFICIENCY KNOWN AS FACTOR X DEFICIENCY (FXD).

CURRENT TREATMENT

It is recommended that people with FXD be seen by a hematologist who specializes in bleeding disorders.

Bleeding in FXD can be treated and prevented with a plasma-derived FX concentrate. FXD also can be treated using plasma-derived concentrates containing the vitamin K-dependent factors (II, VII, IX, X), called prothrombin complex concentrates (PCCs). Your healthcare provider will involve you in determining the best PCC product and dose for you. This is because the additional plasma proteins in PCCs are not needed for those with FXD and repeated doses of these products can lead to increased clotting.

Other non-factor products such as fresh frozen plasma and aminocaproic acid or tranexamic acid may also be used in the treatment of FXD. Talk to your doctor to determine which treatment options are right for you.

FUTURE DIRECTIONS OF CARE

Several products looking at re-balancing the coagulation cascade are in research studies now: anti-tissue factor pathway inhibitors, RNA knockdown molecules, and serpin protein therapies. Even gene therapy may one day be available for FXD. Unfortunately, the very rare nature of this diagnosis makes progress slower, but we must continue to push for better care for those with FXD, and all rare bleeding disorders. 💧

This article was written for Educational Purposes and is not intended to be Medical Advice.



734.544.0015

www.hfmich.org

1921 West Michigan Ave.
Ypsilanti, Michigan 48197

UPCOMING 2023 EVENTS

March 10
Community Night
Virtual Date Night

March 21-22
Lansing Days
Lansing Capitol

May 6-7
SpringFest
Ann Arbor Marriott Ypsilanti
at Eagle Crest

Spring 2023
Women's Retreat

FOR MORE INFORMATION AND
OTHER UPCOMING HFM EVENTS,
PLEASE VISIT HFMICH.ORG/EVENTS.

HFM'S UPDATED VACCINE POLICY

HFM continues to recommend vaccinations/
boosters and use of masks as preventative
measures against the spread of Covid-19.
*However, we will no longer require proof
of vaccination to attend in-person events.*



www.hfmich.org



@hfmich



www.hfmich.org/facebook



www.hfmich.org/camp

FOLLOW US!