

CHAPTER

Factors

WINTER 2023



**Midwest
Hemophilia
Association**



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etranacogene dezaparvovec-drlb



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Patient portrayal;
HEMGENIX not intended for women

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FROM THE EXECUTIVE DIRECTOR



MHA has had a wonderful year full of events, programs, and fundraisers as you will see inside the pages of this issue of Chapter Factors. What makes it wonderful? YOU....our bleeding disorders community! You are an amazing group of people to serve. Thank you for participating in programs and activities.

Let me also say a special thank you to those who have volunteered--whether stuffing envelopes in the office, serving on an event committee, or helping at a program/fundraiser--you're time, talent, and resources have made a difference.

Angela Brown

angela.brown@midwesthemophilia.org



MHA offers assistance to our community members experiencing financial hardships who live in our geographical coverage area of Kansas and western Missouri and/or for current patients of the Kansas City Regional Hemophilia Treatment Center.

If you or someone you know is experiencing financial hardship, the program guidelines and application form can be found on our website, midwesthemophilia.org, or by scanning the QR code below.



**MHA
Financial
Assistance**





QUICK NOTES

Kansas Combo

This year we tried something a little different to better steward our funding and serve our Kansas folks by combining Wichita Education Day and Western KS Education Day into one weekend (aka: Dodge City Education Day and Garden City Education Day). By doing this we were able to invite those driving from a distance to enjoy a complimentary Friday night hotel stay in order not to miss all of Saturday's activities.

Forty attendees enjoyed a variety of topics including discussion about "The Clotting Cascade", a little magic with "Putting Yourself Out There" and rap sessions for the guys (Using Fitness to Fight Back) and gals (Go with the Flow). We did not forget the kids-- they had fun making stress balls, going on a scavenger hunt, and playing games. Despite the heat, everyone also enjoyed the group activity at Chicken N' Pickle.

Thank you to Jan York, Myleah Morton, Cody York, Ashley & Alex Denney, Brittany & Monty Banning for volunteering with the kids as well as Melissa Routon for assisting with prep work. You did a fabulous job. Also, thank you to our sponsors for helping make this program possible.

2024 World Hemophilia Day

On April 17, 2024, the global bleeding disorders community will come together to celebrate World Hemophilia Day. The theme of the event this year is



Above and right, our Kansas folks enjoyed an education day that combined Wichita and Western Kansas community members.



"Equitable access for all: recognizing all bleeding disorders". The World Federation of Hemophilia (WFH) vision of Treatment for All is for a world where all people with inherited bleeding disorders have access to care, regardless of their type of bleeding disorder, gender, age, or where they live.

This April, let's celebrate our community and continue working towards a world where everyone—with hemophilia A or B, von Willebrand disease (VWD) or any other bleeding disorder—has access to diagnosis, treatment, and comprehensive care.

Big Shots Golf

We scored big with the weather for Springfield Education Day. Held at BigShots Golf in early November, this annual event provided an opportunity to connect with the community, better understand insurance, and practice our swing in the golf bays. In addition to that, kids played mini-golf, cornhole, and more yard games.

We appreciate Matt Brown and Mareena Snarey helping out with the kids and our sponsors for supporting the community.

**WORLD
HEMOPHILIA DAY**
APRIL 17, 2024
#WHD2024

EQUITABLE

access for all

**RECOGNIZING
ALL BLEEDING
DISORDERS**



Music & Movie BINGO

Laughter, music, and excitement filled the air at MHA's first ever Music & Movie BINGO night fundraiser at Llywelyn's Pub in Lee's Summit, Missouri this past October. This fun spin on traditional bingo allowed guests to jam out to snippets of their favorite songs as well as test their recall of scenes from popular movies. Just like regular bingo, but instead of shouting numbers, music videos and movie clips are played. The DJ continues to play until someone gets 5 in a row, four corners, a diamond, or other announced patterns on their bingo card and calls out, "BINGO! Sweet treats and prizes were awarded to the winners. Thank you to everyone who came out to support this great cause. We were able to reach our goal of \$10,000 which will go towards the chapter's financial assistance and academic scholarship funds. With such positive feedback from attendees, we plan to host this event again in 2024.

The weather was perfect for our community members at the Springfield Education Day, below and right.



Two of our very own MHA youth, Nathan and Brenn, represented our chapter at this year's Gettin' in the Game Championship.

Nathan participated in the swimming competitions, while Brenn shot some hoops on the basketball court.

"I had so much fun at the JNC!," said Nathan. "I loved swimming a lot, making new friends, and even got autographs from cool athletes. Hanging out with my dad was the best, and I especially had fun with all the swim events. The tether thingy was awesome – it's like a big rubber band pulling you back, and guess what? I swam all four strokes with it! Thanks a bunch for sending us, we had a blast!"

Brenn reports, "My overall experience in Las Vegas was very fun and cool seeing all of the different kids with bleeding disorders like me. It was fun doing basketball because I learned different skills I was able to bring back home and use. Also, the meals and snacks were great."





CALENDAR OF EVENTS

JANUARY

- 1:** Academic Scholarship Applications 2023-2024 Open
- 25:** 2024 UNITE Launch Watch Party



FEBRUARY

- 6:** Missouri Advocacy Day 2024: *Missouri State Capitol, Jefferson City MO*
- 9-11:** MHA Board Training Retreat
- 22:** YETI Conference



MARCH

MARCH IS BLEEDING DISORDERS AWARENESS!

- 6 - 8:** NBDF Washington Days 2024
- 22-24:** Men's Retreat, *Old Kinderhook, Camdenton, MO*

APRIL

- 11 - 13:** HFA Symposium 2024, *Indianapolis, Indiana*

MAY

- 4:** Sip! Sip! Hooray for MHA!
A Wine Tasting Fundraiser



JUNE

- 22:** UNITE for Bleeding Disorders Walk Fundraiser
- 29:** Wichita & Western KS Educ Day

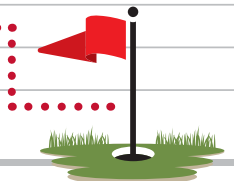


JULY

- 29-Aug 2:** Camp Wilderness

SEPTEMBER

- 12-14** NBDF BDC, Atlanta GA
- 20:** 26th Annual Golf Tournament
- 21-22:** Family Education Weekend



OCTOBER

- 11-13:** FAB Conference
- 25:** Music & Movie BINGO Fundraiser



NOVEMBER

- 9:** Springfield Education Day

MHA Scholarship Applications

Due to changes in filings for FAFSA, MHA's Georgia Northway Academic Scholarship will open December 15, 2023 and close February 29, 2024. Previous winners are welcome to reapply and should receive information directly from the Truman Heartland Foundation who manages this scholarship on behalf of MHA. If you need assistance or have questions about the Georgia Northway scholarship, please contact Rachael Watkins from the Truman Heartland Foundation at (816) 912-4185 or watkins@thcf.org. Scholarship amounts range from \$3,000-\$5,000 with multiple recipients usually being chosen.

The MHA Academic Scholarship application will be available January 1, 2024. Two recipients will be selected to receive \$1,500 each. Submissions should be submitted no later than June 1, 2024.

For additional information, see page 14 for the QR code or visit midwesthemophilia.org, then hover over the "resource" tab to click on "scholarships".

Missouri Advocacy Days Training

Join us and let your voice be heard! Together we can raise public awareness about bleeding disorders and shape public policy that improves the quality of life for all. Your story and experiences play a vital role in the mission and success of our advocacy efforts. We will be advocating for support of All Copays Count legislation with our neighboring chapter, the Gateway Hemophilia Association (GHA). Please visit the MHA website to register by January 15, 2024 or contact the MHA office at (816) 479-5900.

UNITE Launch Party

Watch your inboxes for details on how you can start your own 2024 Unite for Bleeding Disorders walk team during the virtual Unite Launch Party January 25th. We're shooting to reach our goal of 24 teams in 2024!!

FABulous Time for All

The Females And Bleeding (FAB) Conference was hosted by four chapters, Midwest Hemophilia Association, Gateway Hemophilia Association, Bleeding Disorders of the Heartland (Iowa) and the Nebraska Chapter of NBDF (formerly NHF) at Margaritaville Lake Resort in Osage Beach, Missouri, in October with fifty-seven attendees plus speakers and sponsors.

The weekend kicked off Friday evening with a fajita cooking station, fire pits for smores, and friends reconnecting. On Saturday, the ladies enjoyed a couple of interactive welcome activities, a panel discussion with advice and real life tips about surviving day to day life as a woman with a bleeding disorder, followed by a very engaging presentation from Dr. Claudio Sandoval on aging with a bleeding disorder and genetics. Afternoon breakouts included a mental health roundtable and joint health. This year's highlights included water aerobics, a succulent plant activity, and slumber party with pizza, drinks, and games as well as an optional Sunday morning meditation to close out the weekend.



“[FAB] helped me to be more grounded and in the moment. During the succulent activity, the stress that I was previously experiencing melted away.”



“It was great to have a place to be vulnerable and open in a safe place. It gave a few women the chance to share things I think they really needed to get off their chest.”



FEW 2023



MHA's 2023 Family Education Weekend did not disappoint! Held at the Sheraton KC Hotel at Crown Center in September, nearly two hundred of our bleeding disorders family members gathered to experience a conference full of interactive education sessions, time to socialize, exhibit exploration, and loads of fun.

For our keynote presentation, Pinxy Ring, a Puerto Rican rapper and educator from Chicago shared her personal story of self-advocacy and talked about building community through music. Teens and youth explored Science City at Union Station most of Saturday. While on Sunday the Just-Us League made a special appearance to play games, create superhero caps, and read books to the kids about bleeding disorders.

The adults attended breakout sessions with topics such as Von Willebrand A to Z, Spotlight on Siblings, and Understanding Your Health Insurance Options. Some also chose to participate in art therapy and play gene therapy jeopardy. The guys got to use power tools during their breakout session to create a woodworking project as a group while discussing the impact of giving back and "The Ripple Effect." The finished project, shown at right, currently hangs in the MHA office. The Infusion Clinic breakout was also popular for all ages and stages who practiced, instructed, or cheered for those learning the skills necessary to infuse or administer

bleeding disorders medications.

We wrapped things up on Sunday with a gene therapy update presented by Dr. Thomas Cochran from the KC Regional HTC, tons of raffle prizes (thanks to those who donated items and those who bought tickets), and our annual Awards & Recognition Ceremony. Included in the awards and recognition were: academic scholarship recipients, the top Team Captain from the Unite for Bleeding Disorders walk, Big Stick recipients from Camp Wilderness, multiple event sponsors, Volunteer of the Year, Stephen DiCapo, and more.

Be sure to save the date for next year's FEW, September 21-22, 2024! You won't want to miss it!






**Volunteer of the Year
Stephen DiCapo**

Stephen has been an active community member from the early beginning of MHA. This year, he served his community by advocating at state legislation day, participating in the Unite for Bleeding Disorder walk, donating raffle items for our golf & wine fundraiser from his personal collections, volunteering at Camp Wilderness, and even helped organize and implement our first ever wine & bourbon tasting fundraiser. Way to go Stephen!



CAMP WILDERNESS

THANK YOU Camp Sponsors!

TRAIL ANGELS (\$5000-\$9,999)

Children's Mercy 340B
Takeda
Soleo Health

TRAIL LEADERS (\$2,500-\$4,999)

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Bayer
CSL Behring
CVS Health
Genentech
Novo Nordisk
R.A. Long Foundation
Sanofi

DAY HIKERS (\$1,000-\$2,499)

Grifols

LITTLE HIKERS (\$500-\$999)

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Linda Gebauer
Kristin Marema
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Susan Strozewski
Dave Todd
Randall & Heather Ince
Trenton Marema

Thirty-four campers, eleven counselors, two daytime volunteers, eight activity leaders, and eighteen med shed staff gathered at Lake Doniphan Conference and Retreat center this past summer to celebrate the 25th year of MHA's Camp Wilderness in Excelsior Springs, Missouri. With Mario Party-themed games and activities throughout the week, there was no shortage of excitement and fun. Some of the favorites were Mario & Luigi's Pinewood Derby Challenge, Bowser Blaster, Donkey Kong's field games, Mario & Sonic Food Fight, Mario Go-Kart Races, and of course the usual fishing,

crafts, sports, and pool time. We celebrated 25 years with a camp reunion following the close of camp which brought in former volunteers, campers, and medical providers. Thank you to all of our volunteers for helping make camp extra special!

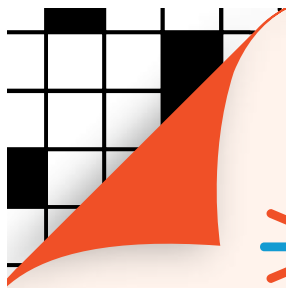


2023

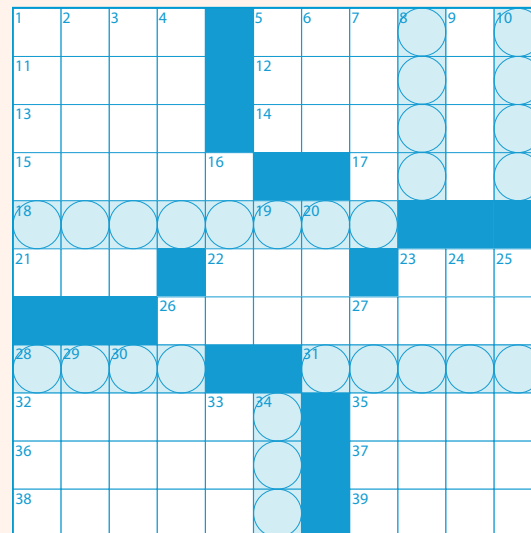


Trenton Marema, camper and Boy Scout, built fishing pole racks and updated a pinewood derby race track for Camp Wilderness as part of his Eagle Scout project. Fantastic work Trenton!





CAN YOU SOLVE **FOR A DIFFERENT** **HEMOPHILIA A** **TREATMENT?** **Test your HEMLIBRA knowledge**



ACROSS

1. Wine barrel
5. Deep fissures
11. Mideast gulf port
12. District
13. Ripped
14. Familiar with
15. Mean
17. Roost
18. The #1 prescribed prophylaxis for people with hemophilia A without factor VIII inhibitors*

*According to IQVIA claims data from various insurance plan types from April 2020 - May 2021 and accounts for usage in prophylaxis settings in the US.

21. Calendar divs.
22. Regret
23. Banquet hosts (abbr.)
26. International travel necessity
28. Check out the _____ treated bleeds data with HEMLIBRA
31. Number of dosing options HEMLIBRA offers

† Number of people with hemophilia A treated as of October 2021.

32. Small hole in lace cloth
35. Central Plains tribe
36. Melodic
37. Towering
38. Reduce
39. Spanish cheers

DOWN

1. Memorable, as an earworm
2. Devotee
3. Medical fluids
4. Prepare to propose, perhaps
5. PC's "brain"
6. Owns
7. Concert venue
8. See Medication Guide or talk to your doctor about potential _____ effects
9. Winter hrs. in Denver and El Paso
10. HEMLIBRA is the only prophylactic treatment offered this way under the skin

16. Pre-Euro currency in Italy
19. Subway alternative
20. Relax
23. Human
24. New Orleans cuisine
25. Mentally prepares
26. Collared shirts
27. Instagram post
28. Ardent enthusiasm
29. Brontë heroine Jane
30. Old Portuguese coins
33. Opposite of WNW
34. More than _____ thousand patients have been treated with HEMLIBRA worldwide†

SOLUTIONS

Across: 1. cash, 5. chasm, 11. Aden, 12. parish, 13. tore, 14. used to, 15. cruel, 17. nest, 18. HEMLIBRA, 21. yrs, 22. rue, 23. MCs, 26. passport, 28. zero, 31. three, 32. eyelid, 35. Oreo, 36. arose, 37. tall, 38. lessen, 39. oles
Down: 1. catchy, 2. adore, 3. serums, 4. kneel, 5. CPU, 6. has, 7. arena, 8. side, 9. MSts, 10. shot, 16. lire, 19. bus, 20. rest, 23. mortal, 24. Croire, 25. steel, 26. polos, 27. photo, 28. zeal, 29. Eyre, 30. Reis, 33. ESE, 34. ten

Discover more at HEMLIBRA.com/answers

INDICATION & IMPORTANT SAFETY INFORMATION

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**.



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.

Your body may make antibodies against HEMLIBRA, which may stop HEMLIBRA from working properly. Contact your healthcare provider immediately if you notice that HEMLIBRA has stopped working for you (eg, increase in bleeds).

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

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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: 12/2021



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Genentech
A Member of the Roche Group

2024 Men's Retreat

MHA & GHA are hosting
an educational getaway
just for the guys!

March 22-24, 2024

@ Old Kinderhook
678 Old Kinderhook Dr
Camdenton, MO



**Midwest
Hemophilia
Association**



Attendees must meet these prerequisites:

- Live in MHA/GHA service areas
- Be 21+ years old
- Have a bleeding disorder and/or
- Be a spouse, parent/legal guardian of someone with a bleeding disorder

**Registration opens in January 2024
at www.midwesthemophilia.org.**

**Registration closes
Tuesday, March 8, 2024.**

Space is limited so RSVP soon!

APPLY FOR MHA'S ACADEMIC SCHOLARSHIPS 2023-2024 Academic Year



**Applications open
January 1, 2024**

Scan this QR code or
visit midwesthemophilia.org
for more information



**Midwest
Hemophilia
Association**





Patient Safety in the Age of Gene Therapy a Central Focus of Review Article

After several decades of preclinical and clinical research, pitfalls and progress, gene therapies for hemophilia A and B have become a reality with recent FDA approvals. While these therapies, along with others still in development, represent both promise and a notable scientific achievement, outstanding questions relevant to long term efficacy and safety remain. Authors of a new review “Hemophilia Gene Therapy: First, Do No Harm,” published in the *Journal of Thrombosis and Haemostasis* (JTH), tackle these concerns.

The authors address fundamental considerations for hemophilia patients, cognizant that each individual has their own personal health goals and comfort level with open-ended questions of long-term risk vs. therapeutic benefits. Safety, in context of the hemophilia communities’ unique history, is a prominent through line in this paper. They acknowledge concerns of the potential for both innate and adaptive immune responses to adeno-associated viral (AAV) vectors and to the possible integration of the given vector into the genome of gene therapy recipients. These types of responses could have safety and efficacy impacts, including inflammatory effects on the liver or the development of tumors or malignancies.

“Gene therapy is a complex biological ‘drug’ for which, despite 30 years of development, there are many unresolved questions, and the unknowns remain top of mind for clinicians and PwH alike. Evaluation of the risks and benefits of any new therapy requires the careful consideration of all the available

information and a shared decision-making approach should be employed, explain the authors. “This is particularly important in the consideration of gene therapy, given the fact that AAV-mediated gene therapy is a one-time irreversible therapy. A fully informed decision must be ensured, and a robust shared decision-making approach is mandatory for these therapies.”

The review summarizes clinical trial data that supported the regulatory authorization of valoctocogene roxaparvovec in Europe to treat hemophilia A and etranacogene dezaparvovec-drlb in Europe and the United States to treat hemophilia B.

“Gene therapy is a complex biological ‘drug’ for which, despite 30 years of development, there are many unresolved questions...”

NOTE that since the publication of this paper, valoctocogene roxaparvovec also received U.S. Food and Drug Administration approval under the brand name ROCTAVIAN™.

The authors highlight initiatives taken by NHF and other organizations to prioritize safety for hemophilia patients who are either considering gene therapy or for those who have received the one-time treatment. A primary example of this is NHF’s submission of a Citizen Petition to the FDA in 2022 requesting that a Risk Evaluation and Mitigation Strategy (REM) be required as a condition of approval for both valoctocogene roxaparvovec and etranacogene dezaparvovec. Read the full petition to learn more.

While the aforementioned products

received FDA approval without meeting the REM requirements, the document’s key elements remain germane to protecting long term patient safety. These include training and education for healthcare providers (HCPs) on gene therapy and its management in patients with hemophilia, with particular emphasis on the central role of shared decision making (SDM). The crucial function of facilities charged with administering gene therapies, specifically federally funded hemophilia treatment centers, is also emphasized.

The authors stress the importance of high enrollment in the World Federation of Hemophilia’s global gene therapy registry, ideally placed to collect adverse event data and other developments from patients who receive these products. Steps towards achieving greater health equity, including broader representation in clinical trial design, is also a focus.

The paper concludes with a series of recommended steps that could be taken within the hemophilia community to ensure the safety and optimal outcomes for PwH who might opt to receive a gene therapy product. These recommendations dovetail closely with the elements of the earlier REM. This article is currently available in a pre-proof PDF version on the JTH website.

NHF also recorded a webinar that serves as an excellent companion resource to the review article. It was presented by lead author Leonard A. Valentino, MD, President, and CEO of NHF.

Lastly, please see this additional paper highlighting a great tool for SDM and gene therapy.

— Valentino LA, Kaczmarek R, Pierce GF, Noone D, O’Mahony B, Page D, Rotellini D, Skinner MW, *Hemophilia Gene Therapy: First, Do No Harm Journal of Thrombosis and Haemostasis* (2023), doi: <https://doi.org/10.1016/j.jtha.2023.06.016>.

Disclaimer: NHF provides periodic synopses of articles published in peer reviewed journals, the purpose of which is to highlight papers that cover a wide range of topics and speak to a broad spectrum of the inherited blood disorders community. Topics include shared decision making, gene therapy, health equity, and more. Any questions about this article should be directed to the publishing journal and/or the study authors. This content is for general information only. NHF and MHA do not give medical advice or engage in the practice of medicine. NHF and MHA under no circumstances recommend particular treatment for specific individuals and in all cases recommends that you consult your physician or HTC before pursuing any course of treatment.

Brittany Slossberg

Relationship builder

About Brittany

Brittany is a Hemophilia Community Liaison who has a passion for creating long-lasting relationships with others. She has built a strong comradery with many patients in the Colorado community.

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The National Hemophilia Foundation Announces a New Name

In 1948, the foundation got its start as simply “The Hemophilia Foundation” – then in 1956, the foundation formally incorporated into what is known today as – the National Hemophilia Foundation. Even then, it was important to capture the organization’s expanding footprint across the U.S.

Now, in 2023, the time for change has come again.

Because of our longstanding name, the National Hemophilia Foundation is best known for helping people with hemophilia and also for serving the individuals and professionals who care for that community.

However, in the foundation’s many decades, we have also long served those facing other blood and bleeding disorders such as von Willebrand disease, rare factor deficiencies, platelet disorders, and more.

Over the past 75 years, our powerful combination of research, education, and advocacy has improved the lives of people and families with a range of conditions – yet our name and image has not reflected that.

Now, as the National Bleeding Disorders Foundation (NBDF), we can be more inclusive of everyone we serve. Although hemophilia remains a major focus, our name ensures that all people with VWD and rare and ultra-rare deficiencies know that they can find a home within NBDF.

Although we’re adopting a new name and a new look, our work will not change in the short term. We remain

dedicated to supporting our network of over 50 chapters across the country and channeling funds into blood and bleeding disorders research. And we will continue to educate and support families with these disorders as we work tirelessly to protect access to health care on the state and local level.

Over the long term, our new name will challenge us to explore how we can harness our resources and networks to help people facing other blood and bleeding disorders – because after all, bleeding disorders are blood disorders. In the same way that the National Hemophilia Foundation evolved to help people facing blood and bleeding disorders beside hemophilia, we can evolve once again to assist more families in need. Many rare blood and bleeding

disorders don’t have a national support and advocacy network like ours — and

together, we have a historic opportunity to change that.

As the National Bleeding Disorders Foundation, we will use our longtime legacy to inspire a future where even more people have access to the treatments and support they need to thrive. We will remain true to our core vision while raising awareness, expanding reach, and continuing to build upon 75 years of history, hope, and progress. With the launch of our NBDF’s rebrand, you may have some questions. Please feel free to visit hemophilia.org for FAQs.

— Source: www.hemophilia.org

What populations will the National Bleeding Disorders Foundation be serving now?

Hemophilia remains a priority for us and we will continue to serve other bleeding disorders including VWD and rare and ultra-rare deficiencies.

To inform the rebranding, we completed phase 1 of the landscape analysis by listening to the populations we currently serve in focus groups, interviews and surveys.

We are conducting phase 2 of the landscape analysis to connect with other organizations to learn about other blood and bleeding disorders. We will use the data from the analysis to determine what programs, services, and education are already in place for other diseases and disorders, so we can collaborate and not duplicate efforts.

Our name has changed, but we are not changing our focus. Our lived experience experts, all family members, chapters, researchers, Hemophilia Treatment Centers, government partners, and industry partners will remain our sole focus.



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Community Members, Lori Moore and daughter, Daytona, along with Executive Director, Angela Brown, attended NHF's 75th Bleeding Disorders Conference held at the Gaylord National Resort & Convention Center in National Harbor, August 17-19, 2023 where an official announcement was made about the newly named, National Bleeding Disorders Foundation. "If you have never been to a [NBDF] conference it's time to start planning to go to one," says Lori Moore. "We got to attend wonderful sessions and learned so many new things. We would like to thank the Midwest Hemophilia Association for the sponsorship for Daytona and I to be able to attend."



STAY CONNECTED!



If you currently do not receive email announcements from MHA, but would like to, please visit our website, click on "**BECOME A MEMBER**" tab where you can update your contact information by completing the member form.

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25TH ANNUAL MHA GOLF TOURNAMENT



Rain, Rain, Go Away, MHA had Golf to Play!

Despite a very rainy day, 24 golf teams and 12 volunteers showed up to play and serve at MHA's 25th Annual Golf Tournament fundraiser at Winterstone Golf Course in Independence, Missouri.

In honor of our 25th year, a cash prize was offered for the "Best Dressed Golfer" which ended up being shared by Kyrie Smith and Danielle Kempker. These ladies took it to the next level and even decorated their golf cart!

Thank you to our players and sponsors for supporting this year's event. With your help, we raised just under \$20,000! Not too shabby for such a soggy day!!



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New Study Looks at Hemophilia Survival Rate Disparities

Results of a new study published in the journal *Haemophilia* are illustrative of persistent health disparities existing within the U.S. hemophilia population.

The data that informed the study was drawn from National Vital Statistics System (NVSS), which generates comprehensive statistics on births and deaths in the United States. The authors looked at the NVSS' 1999–2020 Multiple Cause-of-Death data set to learn more about rates of hemophilia-related death (rHD).

Investigators ultimately identified 3,115 males associated with a rHD. Data showed that approximately 76.1% of deaths occurred in White men (2370), 13.3% in Black men (413), 7.4% in Hispanic men (233), 2.2% in Asian men (69), and 0.9% in American Indian/Alaska Native men (28), while race and ethnicity data were missing in two cases. A review of the data revealed a noteworthy reduction in overall age-adjusted rHD rates for all race and ethnic groups, with a decrease from 1.37 per 1 million males in 1999-2004 to 0.76 per 1 million males in 2015-2020.

This improvement in rHD rates, driven in part by the evolution of treatment and access to better therapies, represents a positive overall trend. However, the most recent decade's worth of data (2010-2020) also revealed a troublesome disparity as Blacks and Hispanics showed a median rHD of 56 years,

putting them significantly behind their white counterparts who experienced a mean age of 68 years.

“Our observation that Black men had a lower median age at death and that HIV continues to be a leading cause of death among Black males with hemophilia listed in their death certificate in both earlier and recent years could signal ongoing survival disparities among Black people with hemophilia and an HIV infection,”

While the data suggest that HIV has a major impact on rHD in Black males, further research is necessary to identify additional inequities contributing to poorer health outcomes in underserved populations.

“Reported haemophilia-death rates improved in males across all race/ethnicities, but rates were higher Black versus White males. Given the inherent limitations of the current study's data source, further investigation of survival rates and disparities in haemophilia are needed,” the authors concluded.

— The study, “Racial and ethnic differences in reported haemophilia death rates in the United States,” was published online in *Haemophilia* on September 23, 2023.

Source: *The American Journal of Managed Care*, September 23, 2023





WE'RE IN THIS TOGETHER.

Thursday 3:24 pm

Practicing yoga with
her mom

Mariana, living with
von Willebrand disease

Not an actual patient

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Takeda is here to support you throughout your journey and help you embrace life's possibilities. Our focus on factor treatments and educational programs, and our dedication to the bleeding disorders community, remain unchanged. And our commitment to adult patients with von Willebrand disease is stronger than ever.

*Not all activities are appropriate for all individuals.
Consult your doctor prior to engaging in any activity.*

 bleedingdisorders.com | 

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