THE COALITION FOR HEMOPHILIA B

SPRING 2024

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

SYMPOSIUM 2024

KIM PHELAN SPEAKS AT CONGRESSIONAL BRIEFING

MEET PUZZLE ENTHUSIAST AND TRIVIA MASTER, ANNA-MARIE

HEMOPHILIA LANDSCAPE

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THE COALITION FOR HEMOPHILIA B

MISSION

TO MAKE QUALITY OF LIFE THE FOCAL POINT OF TREATMENT FOR PEOPLE WITH HEMOPHILIA B AND THEIR FAMILIES THROUGH EDUCATION, EMPOWERMENT, ADVOCACY, AND OUTREACH.

Empowering Our Community: The 2024 CHB Annual Symposium

From April 25th-28th, the hemophilia B community gathered in Dallas, Texas, for the *2024 CHB Annual Symposium*. This year's event, hosted at the Renaissance Dallas Hotel and virtually, was a vibrant celebration of learning, support, and community spirit. With an array of educational sessions, engaging activities, and heartfelt recognition of volunteers, the symposium underscored the power of unity and dedication within the Hemophilia B community.

Thursday April 25th - Connecting Communities

The first day, *Family Day*, was dedicated to strengthening relationships and fostering a sense of unity among participants, focusing on community bonds. Attendees gathered in a series of social meet-and-greet events that featured four additional new affinity groups (50+, Latino, Inhibitor (hosted by Novo Nordisk), and new families. We received enthusiastic participation and positive feedback.

Thursday's Industry Dinner by Sanofi, *Grateful for You*, celebrated their 10 years of commitment to the hemophilia B community. A panel featuring fellow community members CJ, Erica, Mark, Alex, Brian, Alicia and Landon shared their experiences and discussed treatment options, while attendees also enjoyed engaging in fun interactive activities together.

Later, the exhibit hall officially opened its doors, welcoming enthusiastic exhibitors eager to share a wealth of information. While adults filled the room, special efforts were made to involve teenagers, encouraging them to ask questions and learn. A highlight of the exhibit hall experience was the presence of three parades of delighted children aged 3 to 12. They joyfully delivered handmade thank-you notes and drawings to each exhibitor, spreading cheer as they traversed the hall and shared their heartfelt art and messages with our industry supporters.

Friday April 26th - Educating for Empowerment

Each morning began with a choice between a *Tai Chi & Qigong Sunrise* session or a *Family Fitness* morning exercise session, both promoting physical well-being and mental clarity. After these rejuvenating activities, we gathered in the main meeting hall, where our mornings were kicked off by the dynamic Elec Simon (of *Broadway's Stomp*). Elec, renowned for his inspiring music and empowering message, energized and motivated us to gear up for a day of education and connection. His soul-stirring performances uplifted our spirits and ignited our passions, encouraging self-belief and the embrace of life's possibilities.

The second day was packed with educational sessions aimed at empowering attendees through heartfelt storytelling, valuable knowledge, and essential skills. It commenced with a standout moment of the symposium: the inaugural *Volunteer Awards* ceremony, honoring individuals whose contributions significantly strengthened and empowered the community. Coalition leaders Kim Phelan and Wayne Cook, along with staff members Rocky Williams and Erica Garber, announced the winners.

Rick Starks: *Impact Award* for his decade-long commitment to the community, particularly through his passion for Tai Chi, which has benefited thousands. His dedication has left a transformative mark, embodying lasting change and positive influence.

Milinda DiGiovanni: *Heart of Gold Award* for her unwavering compassion and kindness, symbolized by her calming hugs and joyful presence. Her selflessness and warmth have touched countless lives, making her a beacon of love in the community.

Fel Echandi: *Extraordinary Service Award* for his tireless efforts and genuine compassion, recognizing his exceptional contributions that go above and beyond. His commitment to helping others sets a remarkable standard.

Colin Johnson: *Rising Star Award* from eager child volunteer to dedicated leader, Colin received the Rising Star Award for his inspiring journey and role model status. His growth and unwavering support have made him a shining example of emerging leadership.

Valerie Mooney: *Compassion in Action Award* for her consistent empathy and support, highlighting her dedication even in challenging times. Her presence is a cornerstone of the community's strength.

Chris Maddix: *Community Champion Award* for his positivity and advocacy, recognizing his efforts to uplift the community. His infectious enthusiasm and unwavering support embody the true essence of community spirit.


amani
brooks

Friday's keynote address by Mina Nguyen-Driver PsyD, titled *Hope, Humanity, and War Hero*, recounting a family's harrowing escape from Vietnam and their journey with hemophilia. The keynote underscored the importance of community support and resilience.

Following, Dr. Teneasha Washington and Kimberly Haugstad led a panel of patient advisors in a discussion on *Health Equity in the Hemophilia B Community*, providing valuable insights into the challenges and successes within the community. Participants will hear the results from the health equity project that began at last year's symposium. We will discuss the patient survey, focus groups, and individual interview results while also providing participants an opportunity to provide feedback and develop future recommendations. We thank our partners CSL Behring, Pfizer and Sanofi for supporting this important work.

The R*eady for Anything: Building Together for Tomorrow* panel, presented by CVS, emphasized the importance of preparedness and collaboration in facing future challenges. This panel addressed practical resources and solutions in coordinating with health partners, first responders, and government agencies during large scale emergencies considering our implications as a community with chronic conditions.

We ensured a day filled with fun, engaging and interactive sessions. Kevin Harris led a hands-on *First Aid - Wound Care* demo, using special effects theatrical blood and putty, teaching participants how to manage external bleeds effectively. Vanessa Harris hosted a lively *Nutrition Bingo* session, promoting healthy habits and educating through a bingo format on various aspects of holistic health: diet, sleep, stress management, and movement. Jeanette Jones led a session on *Menstruation and Tracking for People with Bleeding Disorders*, reviewing research and practical tools for recognizing and managing heavy menstrual bleeding.

Fernando Reyes, MEdPsy, kicked off our Latino family education track with his session, *Cuerpo y Mente Juntos (Body and Mind Together)* where he presented on the fundamentals of wellness, the immune system, and steps to prevent cognitive decline. Karen Boyd, LMSW, and David Rushlow, LMSW, led one of their highly sought partner's sessions exploring how effective communication














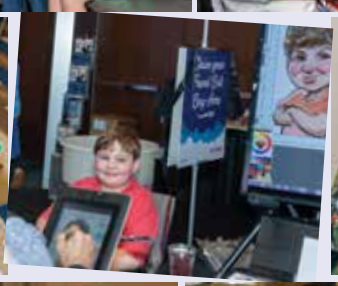





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David
Factor IX level of 37% at 2 years
Patient portrayal; HEMGENIX not intended for women.

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37% AVERAGE FACTOR IX ACTIVITY
ELEVATED AND SUSTAINED FOR YEARS*

GREATER BLEED PROTECTION
VS. ROUTINE FACTOR IX PROPHY†

94% OF PEOPLE DISCONTINUED FACTOR IX PROPHY
AND REMAINED PROPHY-FREE‡

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*Elevated factor IX levels have been observed annually.

†In the clinical trial, annualized bleed rate (ABR) for all bleeds decreased from an average of 4.1 for patients on prophylaxis (prophy) during the lead-in period to 1.9 (54% reduction) in months 7–18 after treatment.

‡Two patients were not able to stop routine prophylaxis. During months 7–18 an additional patient received prophylaxis during days 396–534 (approximately 20 weeks).

IMPORTANT SAFETY INFORMATION

What is HEMGENIX?

HEMGENIX®, etranacogene dezaparvovec-drlb, is a one-time gene therapy for the treatment of adults with hemophilia B who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening bleeding, or
- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is administered as a single intravenous infusion and can be administered only once.

What medical testing can I expect to be given before and after administration of HEMGENIX?

To determine your eligibility to receive HEMGENIX, you will be tested for Factor IX inhibitors. If this test result is positive, a retest will be performed 2 weeks

later. If both tests are positive for Factor IX inhibitors, your doctor will not administer HEMGENIX to you. If, after administration of HEMGENIX, increased Factor IX activity is not achieved, or bleeding is not controlled, a post-dose test for Factor IX inhibitors will be performed.

HEMGENIX may lead to elevations of liver enzymes in the blood; therefore, ultrasound and other testing will be performed to check on liver health before HEMGENIX can be administered. Following administration of HEMGENIX, your doctor will monitor your liver enzyme levels weekly for at least 3 months. If you have preexisting risk factors for liver cancer, regular liver health testing will continue for 5 years post-administration. Treatment for elevated liver enzymes could include corticosteroids.

What were the most common side effects of HEMGENIX in clinical trials?

In clinical trials for HEMGENIX, the most common side effects reported in more than 5% of patients were liver enzyme elevations, headache, elevated levels of a certain blood enzyme, flu-like symptoms, infusion-related reactions, fatigue, nausea, and feeling unwell. These are not the only side effects possible. Tell your healthcare provider about any side effect you may experience.

What should I watch for during infusion with HEMGENIX?

Your doctor will monitor you for infusion-related reactions during administration of HEMGENIX, as well as for at least 3 hours after the infusion is complete. Symptoms may include chest tightness, headaches, abdominal pain, lightheadedness, flu-like symptoms, shivering, flushing, rash, and elevated blood pressure. If an infusion-related reaction occurs, the doctor may slow or stop the HEMGENIX infusion, resuming at a lower infusion rate once symptoms resolve.

What should I avoid after receiving HEMGENIX?

Small amounts of HEMGENIX may be present in your blood, semen, and other excreted/secreted materials, and it is not known how long this continues. You should not donate blood, organs, tissues, or cells for transplantation after receiving HEMGENIX.

Please see full prescribing information for HEMGENIX.

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.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

CSL Behring

HEMGENIX is manufactured by uniQure Inc. and distributed by CSL Behring LLC.
HEMGENIX® is a registered trademark of CSL Behring LLC.

BRIEF SUMMARY OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use HEMGENIX safely and effectively. See full prescribing information for HEMGENIX.

HEMGENIX® (etranacogene dezaparvovec-drlb) suspension, for intravenous infusion
Initial U.S. Approval: 2022

----------INDICATIONS AND USAGE----------
HEMGENIX is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who:
- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening hemorrhage, or
- Have repeated, serious spontaneous bleeding episodes.

----------CONTRAINDICATIONS----------
None.

----------WARNINGS AND PRECAUTIONS----------
- Infusion reactions: Monitor during administration and for at least 3 hours after end of infusion. If symptoms occur, slow or interrupt administration. Re-start administration at a slower infusion once resolved.
- Hepatotoxicity: Closely monitor transaminase levels once per week for 3 months after HEMGENIX administration to mitigate the risk of potential hepatotoxicity. Continue to monitor transaminases in all patients who developed liver enzyme elevations until liver enzymes return to baseline. Consider corticosteroid treatment should elevations occur.
- Hepatocellular carcinogenicity: For patients with preexisting risk factors (e.g., cirrhosis, advanced hepatic fibrosis, hepatitis B or C, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age), perform regular (e.g., annual) liver ultrasound and alpha-fetoprotein testing following administration.
- Monitoring Laboratory tests: Monitor for Factor IX activity and Factor IX inhibitors.

----------ADVERSE REACTIONS----------
The most common adverse reactions (incidence ≥5%) were elevated ALT, headache, blood creatine kinase elevations, flu-like symptoms, infusion-related reactions, fatigue, malaise and elevated AST.

To report SUSPECTED ADVERSE REACTIONS, contact CSL Behring at 1-866-915-6958 or FDA at 1-800-FDA-1088 or *www.fda.gov/medwatch*.

----------USE IN SPECIFIC POPULATIONS----------
No dose adjustment is required in geriatric, hepatic, or renal impaired patients.

Based on November 2022 version

becomes the cornerstone of fulfilling relationships. Laura Echandi, who led two talks for Latino families on parenthood and marriage with hemophilia, is driven by personal experience: her son has severe hemophilia B, and both she and her daughter are carriers. This direct connection fuels her passionate commitment to making a meaningful impact within the hemophilia community.

Friday's Industry Sponsored Dinner, *Disco Trivia by Gut Monkey*, presented by CSL Behring, featured a lively disco-themed game night with interactive trivia spanning pop culture to gene therapy, complete with glow items and prizes for winners. It was an engaging evening of fun and competition!

We concluded the night by hosting an unforgettable, tear-filled screening of Believe Limited's latest film, *On the Shoulders of Giants*. Jane Smith introduced the film and spoke of the pride she feels for Sanofi supporting the film series. This deeply moving documentary celebrates the resilience and courage of our community's legends, capturing Coalition President, Wayne Cook's journey across the country to reunite with fellow blood brothers and sisters. Through reflections on lives lost, the joys of family and friends, and untold stories of how hemophilia shapes their lives today, the film resonated deeply with all of us.

After the screening, Wayne and his blood brothers, Ray Dattoli, Lee Hall, Shelby Smoak, Ray Stanhope and Bobby Wiseman shared an inspiring and heartwarming reflection, leaving us profoundly moved.

Saturday April 27th - Advocating for Change

The final day of the symposium was dedicated to advocacy efforts, amplifying voices on critical issues and catalyzing impactful change within our community. Sessions focused on legal strategies, navigating insurance challenges, and empowering patients to advocate for their needs. Attendees engaged in discussions on relationships, nutrition, and overall well-being, alongside opportunities to interact with medical leaders and experts for the latest scientific insights and advice.

Sanofi presented a lively Industry Sponsored Breakfast featuring Michael D. Pickett-Leonard, PhD, M, discussing the rebalancing approach to hemostasis. This was followed by the always popular Dr. Christopher E. Walsh, Director of Hemophilia at Mount Sinai in New York City, where he offered an intense review of hemophilia B in his keynote presentation including where we are and what you need to know to make well informed decisions.

The *Hemophilia B Landscape* panel featured medical experts discussing the latest research, treatment advancements, and updates on clinical trials, showcasing a variety of new options. Moderated by David Clark, PhD, the panel included Nashwa Choudhry, PhD, Brad Winn, PhD, MBA, and Patrick Yue, MD, who presented advancements in current and upcoming hemophilia B therapies and addressed audience questions. The session covered the significant expansion of treatment options, encompassing longer-acting clotting factors, subcutaneous injections, gene therapy, and more innovative approaches.

Brian Mahony, FACSLM, CEO, of the Irish Haemophilia Society and former President of the World Federation of Hemophilia and European Haemophilia Consortium, moderated the *Gene Therapy Roundtable Discussion*. The session brought together patients and caregivers nationwide. Three gene therapy patients shared their experiences, sparking lively conversations and numerous questions, which we anticipate continuing throughout the year.

Saturday's Industry Sponsored Lunch Symposium, *Your Healthcare Team and You: Shared Decision Making in an Evolving Treatment Landscape*, presented by Pfizer, Inc., featured insights from Mindy Simpson, MD, and Tiffany Smith, DNP, emphasizing the importance of informed decision-making in hemophilia B care. Attendees gained valuable perspectives on current research, enhancing their ability to navigate treatment options effectively.

The afternoon sessions focused on legal and financial guidance and navigating new insurance pitfalls, and self-advocacy. At the *Transitions to Adulthood and Beyond* session led by Donald Akers, Jr., JD, attendees discovered essential tools for safeguarding finances and preserving benefits like Medicaid. Insights into proposed changes to Medicaid limits were provided, empowering families to navigate life transitions confidently and ensure financial stability while securing access to critical benefits. At the *BVoice Insurance IQ* session led by James M. Romano, MPA, MBA, attendees gained valuable insights into navigating insurance complexities, including copay accumulators, alternative funding mechanisms (AFP), and formulary changes. Participants learned actionable steps to enhance their advocacy skills and secure optimal treatment coverage.

Saturday evening closed with the Final Night Dinner Event featuring lively line dancing, entertainers, karaoke, and games that kept the energy high and the laughter flowing. Attendees enjoyed a memorable evening celebrating their time together and creating lasting memories.

Kids and Teen Programs - Fostering Bonds

The symposium's youth and teen camps offered a vibrant array of activities aimed at enriching young attendees' experience. Children delved into artistic expression, unleashing their creativity with various projects. They also personalized their spaces with their artwork and original creations, adding their unique touch to the event spaces. Activities like the *Move &*



Groove Challenge and the *Prickly Pear Cactus Course* not only fostered friendships but also sharpened problem-solving skills through engaging challenges.

Interactive workshops, such as Elec Simon's drumming session, combined music with empowering messages of discipline and community spirit, resonating deeply with participants. In the *Trail Blazin' Bs* activity, children created artwork and heartfelt cards for the hemophilia B community, spreading positivity and support. Practical sessions like Darlene Shelton's *Emergencies Happen* equipped kids with essential safety planning skills, while discussions on *Infusion with Hope* and the *Power of Kinesiology* promoted healthy habits and self-care techniques.

At the *Leading Edge* program presented by Pfizer, GutMonkey engaged teens with lively symposium sessions on building communities, teamwork, understanding bleeding disorders, and shared decision-making. The program balanced informative discussions with laughter, interactive activities, and opportunities to connect with new friends, ensuring a dynamic and enjoyable experience tailored specifically for teenagers.

Parents emphasized the program's role in nurturing connections and understanding among families, highlighting its profound impact on young attendees. "It was great being able to meet and talk with other families," shared one parent, emphasizing the importance of these activities in fostering connections and understanding among participants.

Participant Reflections:

Participants shared overwhelmingly positive feedback, expressing gratitude and highlighting the Symposium's impact."

The sentiment of appreciation was echoed by many, including a parent who said, "Thank you so much! We had a blast and really enjoyed hanging out with all the families and old and new friends. Thank you for showering so much love and praise on Chris for his birthday. He sometimes feels isolated and fearful because of hemophilia but you guys reminded him that he's not alone. David and I salute you for your compassion."

Another participant reflected on the profound impact of the event, stating, "From the bottom of my heart, I want to thank you for not a great symposium, but for a wonderful experience everyone on your team arranged for the community. Myself, having a background in events as a Brand Manager, I have to tell you that you guys nailed everything. From top to bottom. From before the event to after the event. As a minority, coming from Puerto Rico, it was more than a blessing coming to this symposium."

And a few more…

"Thanks to the support of our industry partners and friends, you have brought this symposium to new levels. From the presentations, topics, support, and family time; it was the best symposium by far. Matt and I are so grateful we could come this year. I love watching him pave his own path in the community as he makes new friends and feels a fellowship that soothes his soul. I hope you all know the impact you make to each one of us. Your efforts meet each person where they are in their journey, and somehow pull us all In, close. Thank you so much!"

"If you have hemophilia B and you weren't able to attend the meeting, reach out to someone at The Coalition for Hemophilia B about attending a future program. This organization is truly meeting the needs of the hemophilia community and touching the hearts and souls of people they serve and their clinical and industry partners! Thank you for what you have given us all!"

"Kim Phelan and her team of dedicated, connected and passionate leaders knocked it out of the park this year and delivered an annual meeting to remember."

"Kim, you and your team did a fabulous job this week. You have tirelessly worked over three decades to expand and grow the Coalition. You have officially ushered the Coalition into the second largest



conference spot in the USA and have nurtured the mental health within the community, giving us a safe space to gather, be vulnerable, share stories, gain new knowledge, engage with industry and make life lasting memories. The Coalition now feels like the strongest hemophilia support network I'm familiar with and I gush with pride for you and your team because your work is your passion and your passion is the people. I honestly can't express all my emotions from this meeting, just know it's a complete honor to see the community getting the love, hope and education needed to navigate life's challenges...Thank you!"

"Thank you for having us! My son left there so much more confident and is actually looking forward to his next infusion! Thank you all for welcoming us into the community! Look forward to seeing everyone next year!"

Until Next Year!

The 2024 Symposium was a memorable event, filled with valuable insights, meaningful connections, and engaging activities. It highlighted the strength and resilience of the Hemophilia B community, celebrating the collective spirit of support and empowerment. As one participant aptly summarized, "Words can't express what this now means to us each year... Meeting just one new family each time has impacted our family so much! Thanks so much to everyone."

The Coalition for Hemophilia B wishes to express our gratitude to the many sponsors and exhibitors without which our 2024 Symposium would not have been possible. These include:

We also want to thank our many speakers, volunteers, and staff team members who made the whole event happen.

Please join us at our 2025 Symposium in Orlando and at other programs and events throughout the year. Stay tuned for more information on our 2025 Symposium in Orlando, Florida! More information will be available over time on the B Hub and the CHB website: www.hemob.org.

READY TO DISCOVER WHAT COULD BE NEXT IN HEMOPHILIA TREATMENT?

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Pfizer

ADVOCACY NEWS

CHB COO KIM PHELAN SPEAKS AT CONGRESSIONAL BRIEFING HOSTED BY THE INSTITUTE FOR GENE THERAPIES (IGT) AND ALLIANCE FOR REGENERATIVE MEDICINE (ARM)

BY GLENN MONES

May 2024, CHB COO Kim Phelan represented our community speaking at a Congressional briefing on how changed to the Medicaid Rebate Program will increase barriers patients face to accessing cell and gene therapies. The panelists discussed which policies are needed to improve patient access, such as enabling value-based payment arrangements, and addressing cross-state Medicaid barrier. The briefing also addressed patient and advocate perspectives on how changes could impact patient access.

IGT and ARM brings together experts from across the healthcare system as well as patients and others to advocate for a modernized policy framework that encourages innovations and promotes patient access to the treatments we and others need. Kim's presentation was coordinated with the assistance of James Romano, CHB's advocacy and policy "man in the field."

Kim had the opportunity to speak about the importance of gene therapy to our community, with a special emphasis on access and reimbursement. Kim, along with other panelists and IGT members, made a visit to key legislators on Capitol Hill later that afternoon.

Earlier that day, Kim and Jim also went separately to meet with legislators to speak about having them sign the *Help Copays Act (H.R. 830*).

Stay tuned for more information about this issue as it becomes available. You can always check the B Education Hub at https://www.hemob.org/education or the CHB website at https://www.hemob.org/.

women & girls with hemophilia

articles to support, educate, and empower

Meet Puzzle Enthusiast and Trivia Master, Anna-Marie!

BY SHELLY FISHER

"My head is filled with useless information," Anna-Marie says with a smile. Attributing her self-assessment to a love of puzzles, trivia, history, social science and an avid love of books.

Sharing that her penchant for solving puzzles didn't stop with table-top jigsaws, Anna-Marie confided that she works daily on word puzzles like cryptograms. Based on her role as "the organizer" in her home where she is responsible for stocking the fridge and "making things fit," it's no surprise that the well-known, old-school video game, Tetris, takes up space on her phone.

Currently reading a fantasy novel, *The Night Angel* by Brent Leaks, Anna-Marie recalled two all-time favorites – *Room* by Emma Donahue and Gazebo by Emily Grayson. Why did she select those titles? "They're both fiction, but the way they're both written…it makes you feel as if everything is happening to you."

Her love of history started in the 5th grade, and she shared that she has a particular interest in World War II and the depression era. "With any great conflict, there's always going to be things that are interesting, but with this era, you see people acting out of heroism and depravity."

Anna-Marie felt that her strongest knowledge base was in the social sciences arena, and more specifically the area of anthropology. She also shared that she is intrigued by the Roman and Greek myths, in addition to documentaries detailing evidence of the cities of Troy and Atlantis.

In her free time, the puzzle enthusiast/trivia master can be found window-shopping, walking through antique stores, and thrifting with family and friends. One of her favorite purchases was an old sewing machine that fit into its own table. When asked how her family and friends might describe her, she offered, "someone who is blunt, honest, a peacemaker, great speller, and their personal on-call, tech support."

When our conversation turned to hemophilia B, I learned that Anna-Marie has three nephews with hemophilia. She revealed that she had initially been given a diagnosis of blood cancer by her pediatrician,

which was not true, so she and her mom headed to St. Jude's for further testing. It was there that they discovered she has hemophilia B and was in fact, not just a carrier. She was referred to a hematologist who she sees to this day, and who prescribed Amicar for her. Though her levels vary, her percentage hovers around the moderate range and she experiences regular muscle and joint bleeds. She shared that she felt great gratitude towards her mom for supporting her throughout her diagnosis and to her grandmother for being the researcher in the family.

She has some great advice for someone who has just been diagnosed. "Don't freak out. It's not the end of the world. As long as you're careful and knowledgeable, you



can live a pretty normal life. Be part of the community, not just for the sense of not being alone, but also learning a lot. You not only learn tips and tricks from other people, but when they do the seminars on research and trials. If you work somewhere where it's a concern, or your kids are in school, you want to advocate and let everyone know what to do in that situation."

Though she had logged on for several virtual symposiums previously, it was when Anna-Marie attended the Coalition for Hemophilia B's 2021 Symposium in Orlando, Florida, that she and her family were impacted the most. "There was a seminar on mental health and how everyone processes things differently. When you get upset, you're really just thinking differently than others. That helped me a lot."

When asked how the CHB had specifically contributed to her journey with hemophilia B and her family's endeavor to support her, she commented, "It's opened doors that weren't there before with learning and community but also with the assistance they provide so we can attend events, and meeting people you can talk to throughout the year. It made us more knowledgeable and made us feel like we can address it in better ways."

Anna-Marie has learned a lot about herself since her diagnosis. "I'm not a superhuman. I have limitations. I try to do a lot, so it reminds me that I can't do everything. I'm not a she-hulk. I gotta think about what I can and can't do."

When asked to share words that she lives by, Anna-Marie expressed her love for the Book of Proverbs. She smiled and said, "I always go back to 1 Corinthians 13:4. Love is patient, love is kind. It does not envy, it does not boast, it is not proud." She asserted further, "I always try to better myself, whether it's patience, or learning, or improvement."

SHARE YOUR STORY

Are you ready to share your story and help others? Whether you have an incredible career, an extraordinary family, or a tale of triumph, we want to hear from YOU! You will collaborate with an in–house writer to help you communicate your story in a compelling and meaningful way. The best part is that no previous writing experience is necessary! To add your voice and share your insights with The Coalition for Hemophilia B, please contact us at contact@hemob.org.

HEMOPHILIA LANDSCAPE *UPDATES*

BY DR. DAVID CLARK

Spring 2024: A number of the items below were presented at the annual congress of the European Association for Haemophilia and Allied Disorders (EAHAD), February 6 – 9, 2024 in Frankfurt, Germany. Copies of the abstracts (summaries) for the presentations are available for free at https://eahadcongress.com/abstracts/abstract-publication/.

Prophylaxis for Mild or Moderate Hemophilia B

2/9/24 Patients with mild or moderate hemophilia can also develop joint damage despite not having apparent joint bleeds. A group of researchers from Spain decided to see how routine prophylaxis would affect their bleeding responses and their quality of life. Four moderate hemophilia B patients plus one mild B from their treatment center agreed to start prophylaxis with extended half-life factor IX, once a week at 60 IU/kg. All of the subjects had been on on-demand treatment and all had established joint damage of various severities. Their average annualized bleeding rate (ABR) fell from 3.0 before starting prophy to 0.25 after. The authors also looked at five hemophilia A patients and found similar results. Despite having to infuse more often, the patients reported satisfaction and improvement in their quality of life. [EAHAD abstract PO083]

Postpartum Hemorrhage in Women with Bleeding Disorders

1/24/24 Postpartum hemorrhage (PPH: bleeding after giving birth) causes almost one-quarter of all maternal deaths worldwide and is the leading cause of maternal mortality in both low-income and developed countries. Women with bleeding disorders like hemophilia and von Willebrand disease are at increased risk. A group of researchers in Alberta, Canada looked at all births in the province between 2010 and 2018 (311,330 women with a total of 454,400 pregnancies that resulted in live births).

They found that women with bleeding disorders had about 2.3 times the risk of PPH than did the women without bleeding disorders. They also had about 4.7 times higher risk of severe PPH. In addition, the women with bleeding disorders had about 2.9 times the risk of antepartum hemorrhage (bleeding during pregnancy, before giving birth) and received about 2.8 times more blood transfusions. There was a higher risk of PPH, even for women with factor levels above 50%.

The startling fact is that only about half (49.5%) of the women with bleeding disorders had their factor levels checked during the third trimester before birth. This was the case whether the women had a previous diagnosis of a bleeding disorder or not. However, there were no significant differences between women with bleeding disorders and those without when it came to the baby's survival and health.

The authors conclude that in spite of comprehensive care in women with bleeding disorders, they are still at increased risk of adverse pregnancy outcomes compared to women without bleeding disorders. [Alam AUI et al., Haemophilia, 30:478-489, 2024]

Mental Health of Carriers in China

4/2/24 A group of Chinese researchers looked at 127 hemophilia mothers (93 As, 34 Bs) at their hospital. They found a median clotting factor level (factor VIII or factor IX) of 74% of normal (range 9 – 174%) compared to a median factor level of 149% (range 93 – 189%) in the control group of patients who are not carriers. Of the carriers, 14.3% had factor levels below 40% and had hemophilia, although some carriers with higher levels also had bleeding symptoms. On a standardized survey, 67.7% of the carriers had psychological symptoms. Obsessive-compulsive disorder (OCD) was the most prevalent disorder diagnosed and was sometimes severe. The authors point out that "it is critical to develop efficient strategies to improve psychological well-being." [Wang W et al., Curr Med Sci, online ahead of print 4/22/24]

Bleeding in Young Hemophilia Carriers

2/8/24 Two papers presented at the European Association for Haemophilia and Allied Disorders (EAHAD) annual meeting looked at the characteristics of bleeding in young carriers (up to age 18). The first study looked at girls in the PedNet registry, a collection of data from hemophilia A or B patients in 19 countries who have factor levels of 25% or lower and were born on or after January 1, 2000. The 23 girls who were included had a median age of 10.1 years. The girls included four severes (3A, 1B), four moderates (2A, 2B) and 15 milds (10A, 5B). Joint bleeds occurred in 75% of the severes, 50% of the moderates and 13% of the milds. The median age at first joint bleed was1.3 years for the severes, 5.4 years for the moderates and 6.3 for the milds. Five subjects were on prophylaxis (3 severe, 2 moderate). In summary, a significant number of the girls had joint bleeding and needed prophylaxis. It is important to screen girls in hemophilia families early in their lives. [EAHAD abstract PO162]

A similar study from Ireland looked at 217 women (115 As, 62 Bs) with a median age of 9.6 years. The women were from hemophilia families, but not all were carriers. Using 40% of normal factor levels as the cutoff between hemophilia and normal, they found 78.3% of the subjects were in the normal range, 47.5% were in the mild range and 0.5% (one subject) was in the moderate range. There were no subjects with severe hemophilia. (Note that internationally, the upper cutoff for hemophilia is a factor level of 40% of normal. However, the U.S. uses 50% as the cutoff. Therefore, in the U.S., there probably would have been more milds with levels between 40 and 50%.)

Only 17% of the subjects received treatment with the majority of those receiving antifibrinolytics and/or DDAVP (for As only). Only 11% of the As receiving treatment took factor VIII and only 4% of the Bs receiving treatment took factor IX. Overall, 20% of the A carriers received treatment compared to only 9% of the Bs. The average age of the girls receiving treatment was 12 years, compared to 9.6 years for the non-treatment group. Some of the non-treatment group will presumably receive treatment as they grow older. The authors conclude: "Our novel data from a large paediatric hemophilia carrier cohort highlight significant bleeding phenotype and treatment burden in these children." [EAHAD abstract PO067]

Force-Sensing Treadmill to Detect Arthropathy

3/20/24 A group of Japanese researchers is studying whether gait analysis (the way people walk) using a force-sensing treadmill can be used to detect hemophilic arthropathy (joint damage). They looked at a group of twelve people with hemophilia who have arthropathy, 28 with hemophilia but without arthropathy and twelve people without hemophilia. They found significant differences among the groups. For instance, looking at gait speed, they found an average speed of 3.1 km/h for the non-hemophilia group, 2.0 km/h for the hemophilia without arthropathy group and 1.5 km/h for the hemophilia with arthropathy group. The treadmill also records a number of other gait-related aspects such as the weight on each leg, where each foot strikes the treadmill, wobble both side-to-side and front-to-back, etc.

The most important finding is that people with hemophilia but without known arthropathy had gaits that differed significantly from those for people without hemophilia. Thus, the force-sensing treadmill might provide a way for early detection of joint problems, even before arthropathy can be detected by imaging studies such as MRI or ultrasound.

The researchers also compared results from the force-sensing treadmill with those from the Hemophilia Joint Health Score 2.1 (HJHS), a common tool for the diagnosis of joint issues in hemophilia. The HJHS was designed to identify the initial stages of joint dysfunction among people with hemophilia in childhood and adolescence and has been established as a dependable assessment tool. They found no correlation between the HJHS and the treadmill, except for gait speed. This is probably because the HJHS, as useful as it is, is not sensitive enough to detect the really early changes that can be seen with the treadmill. More work needs to be done on the treadmill before it becomes a useful, convenient tool for analyzing joint health, and the group is continuing their studies. [Mawarikado Y et al., Haemophilia, online ahead of print 3/20/24]

Joint Bleeds in Mild Hemophilia

1/19/24 Mild hemophilia (factor levels between 5 and 50% of normal) has not usually been considered to involve spontaneous joint bleeds or joint damage. A 2018 study among the U.S. Hemophilia Treatment Centers (HTCs) predicted that the annualized joint bleeding rate (AJBR) for a 25- to 44-year-old male with a factor IX level of 20% would be about 0.5. That is, he would only have about one joint bleed in two years. However, a 2012 Canadian study using MRI found damage in more than 30% of joints that had no previous history of bleeds. Thus, the concern is that people with mild hemophilia may be accumulating joint damage, even when they have no obvious bleeds.

A study looking at 70 mild hemophilia patients (57 males and 13 females) from the Yale HTC in Connecticut found 20 (28.6%) patients with a history of joint bleeding, 13 traumatic bleeds (bleeds caused by trauma) and seven spontaneous (with no apparent cause) bleeds. The average age at their first joint bleed was 20.8 years (range 4 to 58). Ten patients developed bleeds between the ages of 10 and 20. The most common locations of bleeds were the knee (11 patients) and the ankle (7 patients). For unknown reasons, three-quarters of the patients with HCV had joint bleeds.

The bottom line is that about 15% of mild patients had abnormal joints, even without a history of joint bleeds. This shows the need for ongoing evaluation of joint health in people with mild hemophilia. [Chiara JB et al., Haemophilia, online ahead of print 1/19/24]

Does Prophylaxis Help Prevent Joint Damage?

2/19/24 With the increasing use of prophylaxis, the incidence and severity of joint damage has decreased. A group of researchers from The Netherlands has looked at this in more detail. They studied X-rays of 1064 joints in 363 patients with severe or moderate hemophilia A or B at their center. Seventy-seven percent of patients with severe hemophilia developed arthropathy (joint damage). However, when the patients were divided by birth cohort (born before 1970, born between 1970 and 1980, born 1981 to 1990, or born after 1990) they could see the decreasing incidence of joint damage.

Factor concentrates were first developed in the 1960s and by the 1970s patients in Europe were using prophylactic treatment. (Prophylaxis started later in the U.S.) In addition, the median age for starting prophylaxis decreased from 18 years to 2.1 years over time. (Note that over time, as the younger cohorts continue to age, their joint damage may increase.)In severe patients, ankles were the most common affected joint, followed by elbows and knees. In the "before 1970" cohort, the incidence of joint damage in at least one ankle was 97.8%. This was reduced to 23.5% in the "after 1990" cohort. Knees were affected the least but still saw a decrease from an incidence of 86.7% in the "before 1970" cohort to 3.9% in the cohort born after 1990.

Moderate patients also saw a decrease in joint damage but to a lesser extent, probably since fewer moderate patients are on prophylaxis. Only 26.1% of moderate patients were on prophylaxis compared to 94.3% of the severes. Interestingly, the most affected joint for moderates was the knee, which was the least affected for severes. The study also had a small number of inhibitor patients in the severe group. Since the development of inhibitors tends to happen in the first 50 exposure days of treatment, the age for first inhibitor development has fallen from a median of 17.4 years for the oldest cohort to 1.5 years in the youngest. Overall, though, inhibitor development did not appear to be associated with any increase in joint damage.

This study emphasizes the importance of prophylactic treatment. Prophylaxis is regular, continuing treatment with clotting factor, as opposed to on-demand treatment in which you infuse only when a joint bleed has already started. [van Heel DAM et al., Res Pract Thromb Haemost, 8(2), 102335, 2024]

Association of Joint Status and Pain with Depression, Anxiety and Stress

2/8/24 Can prophy that keeps your joints happy also make you happier? A group of German researchers looked at the effect of joint status and pain on depression, anxiety and stress using a mental health questionnaire. They assessed the 379 patients for pain, pain persistence and pressure pain thresholds (PPT: a measure of how much pressure can be put on an infected joint before it starts to hurt). They also used the Hemophilia Joint Health Score 2.1 (HJHS) to evaluate the patients.

What they found is not surprising, the worse a patient's pain and joint scores, the worse their mental health scores. Therefore, it is of significant importance to take care of your joints. [EAHAD abstract PO141]

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“I’m embarrassed to

CALL IN SICK BECAUSE OF A BLEED.”

— Julian

Person living with hemophilia

Why is balance so important in hemophilia?

When there’s an imbalance between procoagulants (ie, factor VIII or IX) and anticoagulants like antithrombin and TFPI, your body can’t produce sufficient thrombin to achieve hemostasis.

Discover how some current and potential approaches may help restore hemostasis balance.

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TFPI=tissue factor pathway inhibitor.
This patient story reflects the real-life experience of an individual diagnosed with hemophilia. Individual experiences may vary. This patient was compensated for their time creating this content.

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HEMOPHILIA LANDSCAPE EMERGING THERAPIES

BY DR. DAVID CLARK

Spring 2024 - There is a huge amount of new product development going on in hemophilia B. The potential new products can be separated into three categories, 1) improved factor products, 2) rebalancing agents and 3) gene therapy. These updates are divided into those three categories. Within each category, the entries are generally listed in order of the names of the organizations developing the product. A number of the items below were presented at the annual congress of the European Association for Haemophilia and Allied Disorders (EAHAD), February 6–9, 2024 in Frankfurt, Germany. Copies of the abstracts (summaries) for the presentations are available for free at https://eahadcongress.com/abstracts/abstract-publication/.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products that most people with hemophilia B are currently using, also including products for inhibitor treatment. The improvements include longer half-lives and delivery by subcutaneous injection. This section also includes updates on some of the current products on the market.

FDA Approves Pediatric Indication for Ixinity

3/22/24 Medexus Pharma's Ixinity received approval from FDA to expand their indication to include children younger than 12 years of age. The indication includes on-demand treatment, prophylaxis and use during surgery. Ixinity is now approved for all patients with hemophilia B. The approval was based on a Phase III/IV study of use in children. [FDA approval letter to Medexus 3/22/24]

Ixinity is slightly different from the other standard half-life factor IX products on the U.S. market. BeneFIx and Rixubis both have a factor IX protein that includes the amino acid alanine at the 148th position in the protein chain, designated ala148. (A protein is a long chain of amino acids strung together.) Ixinity has the amino acid threonine (three – o – neen) in the 148th position, designated thr148. These are called isoforms, and both appear in plasma. Different populations have different relative amounts of the two isoforms. Because they are purified from large pools of plasma, plasma-derived products like Alphanine contain both isoforms, and often others.

In the laboratory, there is no significant difference between the two isoforms in terms of bioactivity, but in patients in real life, there does appear to be some difference in some people. Some people seem to respond better to Ixinity and others are better with BeneFIx/Rixubis. For most people, though, all three products work fine. Therefore, if you are not getting good results with your factor IX product, you might want to talk to your doctor about trying a product with the other isoform. [Graham JB et al., Am J Hum Genet, 42:573-580, 1988]

Do Recombinant Products Cause Higher Levels of Inhibitor Development?

2/8/24 The SIPPET study published in 2016 caused a huge stir in the hemophilia community because it suggested that patients on plasma-derived factor VIII (for hemophilia A) products developed only about half the inhibitors as patients on recombinant products. However, there has been little follow-up to that study, which also did not include hemophilia B. An international group of researchers presented a new study at the European Association for Haemophilia and Allied Diseases (EAHAD) annual meeting.

In 1219 hemophilia A PUPs (previously untreated patients), they found an overall incidence of inhibitor development of 26%. The study confirmed the SIPPET results but found smaller differences between plasma-derived and recombinant products. The patients on plasma-derived products had a 20% chance of developing an inhibitor, while those on standard half-life (SHL) recombinants had a 27% chance. Extended half-life (EHL) products came between those at 22%.

In 173 PUPs with hemophilia B, there were no significant differences among the products. Plasma-derived products had an 11% risk of inhibitor development; SHL products came in at an 8% risk and EHL products were at 7%. Although the averages for B appear different, there was a large amount of overlap in the results, so no significant differences were observed. [EAHAD abstract PO129]

REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so the blood clots when it should and doesn't clot when it shouldn't. The clotting system is a complex system of clotting factors that promote clotting and anticoagulants that inhibit clotting. In a person without a bleeding disorder, the system is in balance, so it produces clots as needed. In hemophilia, with the loss of some clotting factor activity, the system is unbalanced; there is too much anticoagulant activity keeping the blood from clotting. Rebalancing agents mainly reduce or inhibit the activity of anticoagulants in the system. Most of these agents work to help restore clotting in people with hemophilia A or B, with or without inhibitors.

Centessa Presents Updates on SerpinPC

2/9/24 and 5/13/24 Centessa is developing SerpinPC, an inhibitor of the anticoagulant activated protein C (APC) as a rebalancing agent. SerpinPC is a biweekly or monthly subcutaneous injection for treatment of hemophilia A and B patients, with or without inhibitors. Centessa presented updates on their Phase IIa clinical studies at the American Society of Hematology (ASH) meeting in December and at EAHAD.

Over 2.8 years, they found a significant decrease in annualized bleeding rate (ABR), which declined from a median of 35.6 before treatment to 1.0 after SerpinPC. Note that the pre-treatment ABR was high because the subjects were all treated on-demand rather than with prophylaxis before the study (at baseline). All 20 subjects (16 As, 4 Bs) had target joints at baseline, but only two still had target joints at the end of the study. The product was well-tolerated with no treatment-related adverse effects. They found no evidence of thrombosis (too much clotting). This is the ongoing Part 1 of Centessa's studies. By the end of 2024, they plan to have confirmed the optimum dose and be ready to move to the next phase of the trials. [Centessa press releases, 2/9/24 and 5/13/24]

Novo Nordisk Presents Updates on Concizumab

2/9/24 Novo Nordisk is developing concizumab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) as a rebalancing agent. Concizumab is a daily subcutaneous injection for treatment of hemophilia A and B patients, with or without inhibitors. Novo presented updates on their Phase III clinical studies at EAHAD.

Novo gave an update of the Phase III study results after all patients had been treated for 56 weeks. The subjects included 80 As (HA) and 64 Bs (HB) without inhibitors plus 76 As (HAwI) and 51 Bs (HBwI) with inhibitors. They found median annualized bleeding rates (ABRs) of 0.7 (range 0.0 to 3.0) for HAwI, 1.1 (0.0 – 3.2) for HBwI, 1.7 (0.0 – 4.5) for HA and 2.8 (0.0 – 6.4) for HB. Although it seems that there is a variation among the different groups, the ranges overlap, so the differences may not be significant. FDA had previously put a clinical hold on the studies because of thrombosis in a few patients. After that, Novo revised the protocol and dosing to FDA's satisfaction, and restarted the studies. There were no additional thromboembolic complications from the restart to the 56-week analysis. [EAHAD abstract OR07]

Novo also presented results for concizumab patients who had surgery during the clinical studies. Major surgeries were prohibited in the studies, but minor procedures were allowed. Thirty patients (out of 278 total) had minor surgeries. This included nine As and ten Bs without inhibitors, plus seven A and four B inhibitor patients. Surgery-related excess bleeding occurred in 14 out of 38 total procedures, and eight were treated with factor. Only one bleeding episode was severe. The median duration of bleeding was two days and the average number of factor injections per patient was 1.5. The surgeries included 24 dental procedures. The others included port removal, colonoscopy, joint fusion and urethral augmentation. [EAHAD abstract PO064]

One of the issues with the rebalancing agents is testing patients to determine whether they have effective levels of the product. Since these products don't contain factor VIII or IX, a test for factor levels is not feasible. What is needed is a test for the overall coagulability of the blood. This has been an ongoing problem in hemophilia as scientists have developed advanced treatments for which measurements of factors VIII or IX are not sufficient. One possibility is the rotational thromboelastometry or ROTEM test, which measures the strength and elasticity of a clot as it forms in a blood sample. However, the Novo group found that ROTEM is not suitable for monitoring of clotting potential with concizumab.

Novo is to be congratulated for publishing this. Although scientists are encouraged to publish all of their work, not just the successful parts, it rarely happens that they publish negative results – experiments that don't work. Negative results are still knowledge. Knowing what doesn't work can help us better understand what might work. It also keeps research groups from wasting time exploring the same rabbit hole over and over. [EAHAD abstract PO021]

Pfizer Updates on Marstacimab

2/8/24 Pfizer is developing marstacimab, an inhibitor of the anticoagulant tissue factor pathway inhibitor (TFPI) as a rebalancing agent. Marstacimab is a once-weekly subcutaneous injection delivered via an auto-injector

pen for treatment of hemophilia A and B patients, with or without inhibitors. They presented two papers at EAHAD.

The first paper looked at data from their Phase III study concerning joint health. In 123 patients without inhibitors, they saw significant reductions in joint bleeds, joint status (decreases in the Hemophilia Joint Health Score (HJHS), where a lower result is better) and the number of target joints. (The results are difficult to summarize because they are presented independently from several different studies, but all of the groups saw similar improvements.) [EAHAD abstract PO074]

In the second paper, they looked at the development of anti-drug antibodies (ADAs) against marstacimab, again in patients without inhibitors. ADAs against factor IX or factor VIII are called inhibitors in hemophilia. Other drugs can also have similar antibody reactions to the drug. ADAs are generally divided into two categories, neutralizing and non-neutralizing. Neutralizing antibodies interfere with the performance of the drug, while non-neutralizing ADAs may bind to the drug, but don't interfere with its function. They found that most ADAs were low and transient, that is, they disappear after a while, in this study within about two months. No patients were ADA-positive by the end of the study. ADAs did not appear to interfere with the treatment or change the incidence of adverse events. [EAHAD abstract PO186]

TiumBio Submits Application for Phase Ib Studies of TU7710 for Inhibitor Patients

3/26/24 TiumBio, a Korean biotech, is developing TU7710, a longer-acting activated factor VII (FVIIa) product for treatment of hemophilia A and B patients with inhibitors. TU7710 consists of FVIIa molecules fused to the protein transferrin. This gives TU7710 a half-life about six to seven times longer than NovoSeven. TiumBio is currently conducting a Phase Ia study evaluating the safety, tolerability and pharmacokinetics (PK) of TU7710 in healthy male volunteers (without inhibitors), with results expected by mid-2024.

TiumBio has now submitted a Clinical Trial Application (CTA) for a Phase Ib study. (A CTA is the European version of the US FDA's IND for approval to conduct a study in humans.) The Phase Ib study will continue the safety/tolerability/PK studies in inhibitor patients. It will also look at various dosage levels to determine the dosage for a subsequent Phase II study. [TiumBio press release 3/26/24]

GENE AND CELL THERAPY

Gene therapy is the process of inserting new, functional factor IX genes into the body to allow it to produce its own factor IX. Cell therapy is the transplantation of whole cells that have been modified to perform a specific function such as producing factor IX.

Gene Therapy for Inhibitor Patients?

2/9/24 Hemophilia patients with inhibitors are not eligible for the current gene therapy treatments for hemophilia A or B. Hemophilia B inhibitor patients produce antibodies against factor IX when it is given by infusion. So, what would happen if a B inhibitor patient were given one of the two approved gene therapies? Two scenarios are possible. In the worst case, their body would start producing new factor IX molecules, but the immune system would still try to eliminate the new factor IX from the body. This would set up a "war" between the immune system and the liver, where the new factor IX is produced. This could be a dangerous, even life-threatening, condition. The best case is that the gene therapy would act like immune tolerance induction (ITI) in which an inhibitor patient is given frequent high doses of factor to induce the body to learn to tolerate it. We don't know which will happen.

Biomarin, who produces Roctavian, a gene therapy for hemophilia A, decided to find out. In a Phase I/II study approved by FDA, they gave Roctavian to four hemophilia A inhibitor patients. The first two patients had current inhibitor levels of 3.8 and 2.2 BU/ml, respectively. Patient 1's inhibitor levels increased for twelve weeks, but started down after that point. However, he then developed an inflamed liver that was treated with corticosteroids, the normal treatment for liver inflammation after gene therapy. His inhibitor level rose after that. Patient 2 had an inhibitor level that rose to 20.1 BU/ml at nine weeks, but then declined to <0.6 BU/ml, indicating the inhibitor had been eliminated. At that point, his factor VIII level had risen to 41.7%.

Two other patients, who had inhibitors that were previously eliminated by ITI, had levels of <0.6 BU/ml before gene therapy treatment. Their factor VIII levels rose to 26.2 and 249% after gene therapy. (Note that 249% is too high and might induce thrombosis, dangerous unneeded clotting.) Neither of their inhibitor levels rose after treatment. No serious or severe adverse events were reported in any of the four patients. The patients did not appear to have any different safety responses than the non-inhibitor patients in the Roctavian clinical studies.

This was an early report from an ongoing study in hemophilia A that was presented at EAHAD, but it provides a glimmer of hope for hemophilia B inhibitor patients. Researchers have also seen elimination of inhibitors by gene therapy in animals, but this is the first human experience. Much more research will be required, but one day, hemophilia inhibitors may be a thing of the past. [EAHAD abstract OR10]

CSL Reports on Hemgenix Gene Therapy after Three and Four Years

2/9/24 CSL Behring markets Hemgenix, a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX gene. At EAHAD, they presented updates on patient results after three years in their Phase III study and after four years in their Phase IIb study. From the Phase III study, 52 of the original 54 subjects completed a three-year follow-up. Their mean (average) ABR for all bleeds was 1.52, compared with 4.17 during the 6-month pre-treatment period when they were on conventional prophylaxis. Their mean factor IX levels were 41.5% of normal one year after treatment, 36.7% after year two and 38.6% after year three. (Note that the standard deviations for these values are fairly large, so there is no evidence that the levels are decreasing.) Fifty-one subjects have discontinued prophylaxis, but one subject had his factor IX levels decline to the 2 – 5% range, resumed prophylaxis and his bleeds returned. [EAHAD abstract OR09]

From their earlier Phase IIb study, which included only three subjects, mean factor IX levels were 31% at 6 weeks after treatment, 40.7% at one year, 44.2% after two years, 36.9% at year three and 45% at year four. The ABR was 0.22 at year three and 0.17 at year four. [EAHAD abstract PO038]

CSL Reports on Quality of Life after Two Years on Hemgenix

3/10/24 CSL published a report on the quality of life (QoL) for patients in their Phase III clinical study of Hemgenix after two years. The study used various questionnaires and compared results from six months before treatment and two years after treatment. On the Hem-A-Qol questionnaire, the categories (domains) of Treatment, Feelings, Work/School and Future showed improvement, while the other categories did not. [Itzler R et al., Haemophilia, online ahead of print 3/10/24]

Frontera Reports Pre-Clinical Results for FT-004

5/9/24 Frontera Therapeutics is developing FT-004, a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX. At the American Society of Cell and Gene Therapy (ASGCT) annual meeting, they presented pre-clinical animal data for FT-004. Their key improvement is to eliminate CpG sequences in the new factor IX gene in order to minimize the immune response to their treatment. CpG sequences are a cytosine base followed by a guanine base in the DNA sequence of a gene. Previous research has suggested that CpG sequences activate the immune system, and thus may affect the efficiency of insertion of the new gene.

FT-004 was tested in both hemophilia B mice and in cynomolgus monkeys, giving good results in both. They saw high factor IX expression and no apparent safety issues in the animals. [ASGCT abstract 1088]

Pfizer's Beqvez Gene Therapy Approved by FDA

4/25/24 Pfizer has been developing Beqvez (fidanacogene elaparvovec), a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX gene. They have now received FDA approval to market Beqvez in the U.S. with the following indication:

BEQVEZ is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with moderate to severe hemophilia B (congenital factor IX deficiency) who:

- Currently use factor IX prophylaxis therapy, or
- Have current or historical life-threatening hemorrhage, or
- Have repeated, serious spontaneous bleeding episodes, and,
- Do not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid as detected by an FDA-approved test.

This is essentially the same as the indication for Hemgenix, CSL's competing factor IX gene therapy, with the exception that Hemgenix doesn't have a limitation on pre-existing antibodies to their vector AAV5. At the same time, FDA approved a test for antibodies against the AAVRh74var vector. Neither product may be used for patients with inhibitors or a history of inhibitors, and neither is intended for use in women.

Beqvez was developed by Pfizer and Spark Therapeutics, which is now a subsidiary of Roche. Pfizer has set a list price of $3.5 million, matching Hemgenix. (Hemgenix was once the most expensive pharmaceutical in the world, but this was recently surpassed by Orchard Therapeutics' Lenmeldy gene therapy for metachromatic leukodystrophy, which has a price of $4.25 million.) [FDA approval letter 4/25/24 and Pfizer communications]

Pfizer Presents Updates on Beqvez

2/9/24 Pfizer presented three papers at EAHAD on Beqvez. The first paper looked at health-related quality of life (HRQoL) in subjects who received Beqvez in the clinical studies. The 42 subjects with a median age of

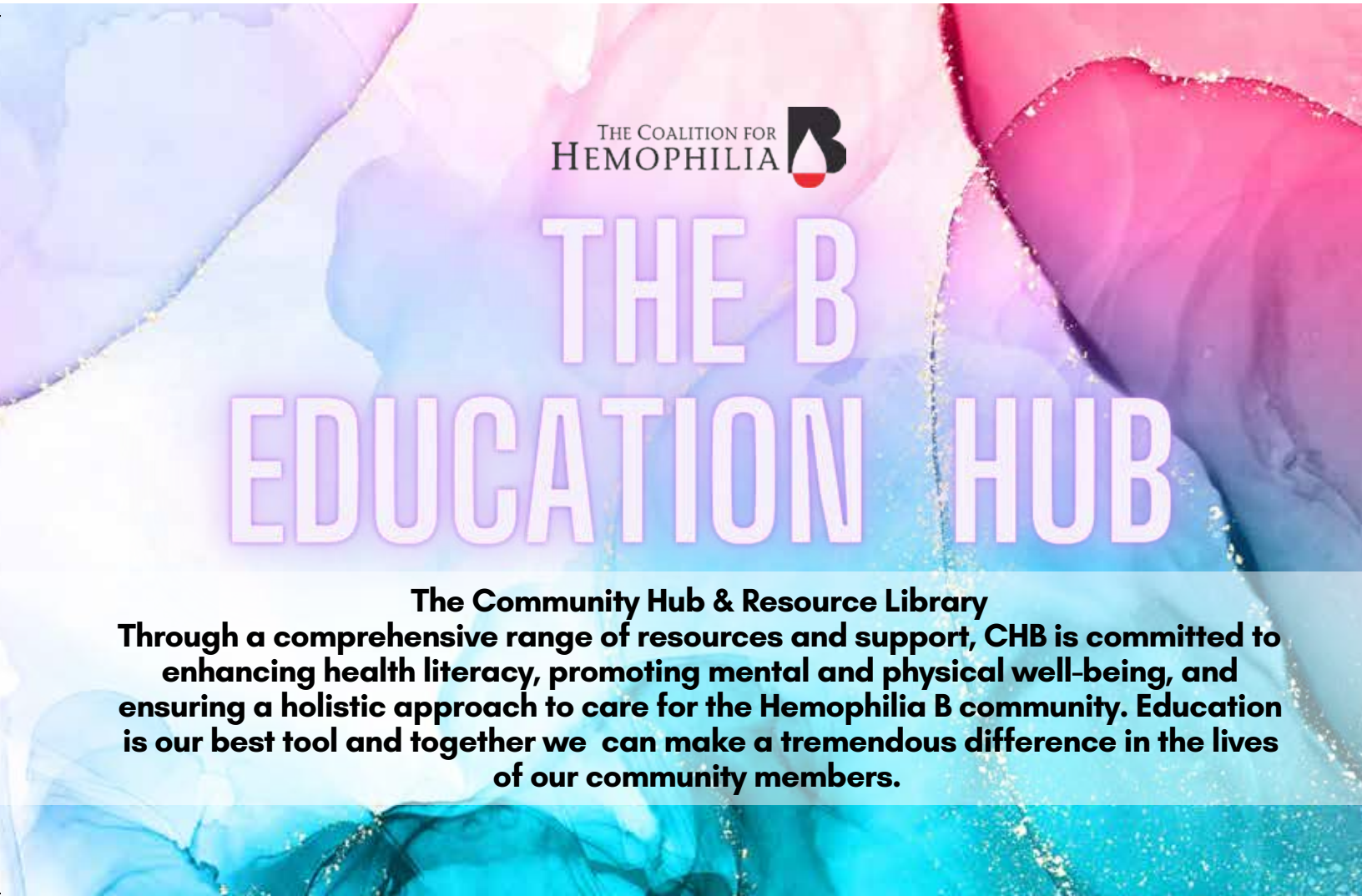
29 (range 18–62) at the time of treatment, completed a number of questionnaires both before treatment and at the one-year mark. On the often-used Haemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) the average drop one year after treatment was 11.2 points, a significant improvement in HRQoL where lower scores are better. They also saw improvements in other aspects including physical health, feelings, view of self, work/school, sport/leisure, treatment and future outlook. [EAHAD abstract PO116]

Pfizer also reported on clearance of the gene therapy vector from the body in the patients in their Phase III study. They looked at vector DNA levels in plasma, peripheral blood mononuclear cells (PBMC, a type of white blood cell), semen, saliva and urine. Complete clearance of the DNA was achieved within an average of 1 – 4 months, except in PBMCs, which took up to seven months. [EAHAD abstract PO132]

Finally, they looked at what doses of an extended half-life (EHL) product like Alprolix would be needed to match the factor IX trough levels provided by Beqvez over the course of 25 years. They assumed that the factor level provided by Beqvez would decline somewhat over the 25 years, a conservative estimate since we don't know how long gene therapy will continue to provide adequate factor levels. For a fixed weekly dosing regimen, the study estimates that Alprolix doses of 251, 174, 117, and 47.8 IU/kg would be required in years 1, 5, 10 and 25. If, instead, the Alprolix dose were fixed at 50 IU/kg, the estimated dosing frequencies would be every 2.72, 3.42, 4.35, and 7.18 days in years 1, 5, 10 and 25. These results demonstrate how high a dose or how often an infusion would be required to match the factor levels provided by Beqvez. This helps to support the cost-effectiveness of gene therapy over traditional factor infusions. [EAHAD abstract PO182]

Regeneron to Continue Work on Gene Therapy for Hemophilia B

3/22/24 Regeneron Pharmaceuticals and Intellia Therapeutics have been developing a gene editing treatment for hemophilia B. The companies received approval for their Phase I clinical study, which is scheduled to begin in mid-2024. However, Intellia has decided to end its collaboration with Regeneron on the project. Regeneron will continue the project on its own. The treatment uses CRISPR/Cas9 technology to insert a new factor IX gene into the genome of liver cells. [Hemophilia News Today article 3/27/24]



HER CARE, HER CHOICE: WOMEN & HEMOPHILIA B

BY ALYSHA MCCABE

***Her Care, Her Choice*, the first of a three-part series, took place virtually on January 31, 2024. The event featured two strong allies and advocates who used their expertise and experiences to guide participants through the latest advancements in the field and share insight specifically relating to women and hemophilia B.**

Susan Salenger, researcher and author of *SIDELINED: How Women Can Navigate a Broken Healthcare System*, joined us to deliver tips and tricks for self-advocacy. She focused on helping women overcome barriers to equal treatment and healthcare. Susan provided ideas for navigating healthcare spaces as a woman, as well as findings from her ongoing research. Susan shared that the best way to be empowered is to try and focus the doctor's visit. This can be accomplished by going into a visit with a written, prioritized list of symptoms, questions, and concerns.

Another important tip is to repeat back what you hear the doctors say. This allows you to be sure that you understood correctly, and it gives the doctor a chance to make sure they meant to say what they did. Salenger will return to share her self-advocacy tips and continue discussions during the remaining *Her Care, Her Choice* meetings on May 9th and October 3rd.

Continuing the meeting, Robert F. Sidonio, Jr. MD, MSc., delved into the essentials of clinical research, providing participants with a foundational understanding of conducting research in bleeding disorders. Dr. Sidonio has been the Associate Director of Hemostasis and Thrombosis at Emory University since 2014. He is a former MASAC member, HFA medical advisor, and Chair of the Women's Health in Thrombosis and Haemostasis.

Dr. Sidonio delivered a concise update on hemophilia carrier nomenclature, shedding light on recent advancements in the field. Additionally, attendees gained insights into optimal outcomes and toolkits tailored specifically for hemophilia carriers, offering valuable guidance on diagnosis and treatment strategies.

One of the biggest takeaways from Dr. Sidonio's presentation was that in medical research and discussions surrounding bleeding disorders, it is important to recognize the unique challenges and considerations specific to women and girls affected. As the community continues to advocate and turn our focus on supporting women and girls with bleeding disorders (WGBD) we face the new challenge of ensuring this group is equally represented in clinical trials.

Our empowerment host, Gha'il Rhodes Benjamin wrapped the evening with her powerful words and strong mindset. Gha'il is a beloved speaker among the community, known for her uplifting message and holding healing spaces for us to connect and express ourselves!

The session concluded with community connection and participants sharing their thoughts about the informative and empowering nature of this event. Thank you to Medexus Pharma for allowing participants to come together and gain access to valuable knowledge and research. The environment was educational, fostered meaningful connections, and provided a safe space for participants to ask important questions relating to women and hemophilia B.

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Comments

"So much great information was shared tonight. Thank you! We are stronger together!"

"Advocate for yourself ladies! You got this!"

"Women matter. We are important. We are hemophiliacs – not just symptomatic carriers."

"Great information about advocating for myself. I have noted what to take to my next appointment."

"Always great to gain more knowledge and spend time with my Blood Sisters!"

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HER CARE, HER CHOICE: WOMEN & HEMOPHILIA B

BY ALYSHA MCCABE

Our Her Care, Her Choice virtual event series returned on March 21st, 2024. The event continued to be an empowering, educational, and eye-opening experience for the attendees.

Participants explored the topic of sexual health and women with bleeding disorders, which left many expressing their gratitude to our passionate and informative speaker for bringing to light topics that are not often talked about.

The night kicked off with a welcome from the program's sponsor, Sanofi. Cindy Bishop, Community Relations and Education Manager for Sanofi, was there to represent the company. She shared that they have created a Spotify playlist of songs inspired by the true sound of hemophilia. A group of men and women got together with writers and music producers to create a collection of music centered around living with hemophilia. You can find the HEM Sessions playlist on Spotify by searching HEM Sessions Vol. 1.

CHB introduced a new, live-stream translation feature during this session, which allowed participants from various language backgrounds to engage with the presentation in real-time. This feature enabled beautiful connections between participants and allowed some of our members to experience the event in a way they were unable to do previously.

Expert speaker, Amber Federizo, APRN, FNP-BC, shared her knowledge and passion for the sexual health of women with an engaging and thorough presentation. Amber educated participants about aspects of female anatomy, menstruation, pregnancy, and sexual function concerning bleeding disorders. She discussed issues surrounding sexual function that can often be overlooked or dismissed as well as provided tips for navigating those situations.

She expressed that beliefs about the prevalence and characteristics of bleeding disorders in women may impede timely treatment. It is important to be knowledgeable about female anatomy and sexual function so that you can advocate for yourself when complications arise. Understanding bleeding disorders in women, identifying symptoms, seeking a diagnosis, as well as classifying disorder type and genetic variant are critical for successful treatment.

Amber discussed various complications, precautions, and steps that can be taken to do things healthfully and safely. She voiced the importance of exercising and strengthening pelvic floor muscles as we age. Amber shared physical therapy tools and trainers that aid in these exercises. When speaking about pregnancy, she stressed how important it is to advocate for yourself. Don't expect to be iron deficient during pregnancy; women should track iron and ferritin levels.

Participants thanked our expert speaker and several expressed how surprised they were to learn so much new information about their own bodies. Many thanks to Amber for allowing community members to have access to this type of educational content that is often considered "taboo."

Following our amazing expert speaker, Gha'il Rhodes Benjamin, an award-winning, Grammy-nominated spoken-word performance-recording artist, joined the group to guide us through a period of community reflection and empowerment. Gha'il did a check-in and asked participants to reflect on their self-care, self-love, and self-worth. Members expressed support for each other as well as shared ways in which they have been working on self-care. Gha'il let everyone know that they should be proud of themselves and left with the words "Continue to take care of yourselves because we women are everything!"

The session was wrapped with participant reflections and raffles. It was clear that participants felt more knowledgeable about their bodies and equipped with new knowledge to advocate for themselves and their own needs. Thank you again to Sanofi, for sponsoring and supporting this engaging and informative programming.

sanofi

Comments:

"Enjoyed getting a chance to learn about what some may consider a "taboo" topic, that needs to be talked about more."

"Thankful for the wonderful educational presentation and I'm shocked at how much I learned."

"What an important and powerful session. I learned a lot about my body and things to consider."

"I am so thankful for the support of the Coalition for having sessions like this!"

COLORADO CHAPTER EDUCATION EMPOWERMENT DAYS

BY ROCKY WILLIAMS

March 2024, we exhibited at the Colorado Chapter of the NBDF *Education Empowerment Days*, a gathering that brought together individuals and families impacted by bleeding disorders. Dr. David Clark and I had the pleasure to connect with numerous families from Denver and the surrounding area. Being a part of this event was truly a joy. We extend our gratitude to the Colorado Chapter, NBDF, and Executive Director Perry Jowsey for their hospitality and for organizing such a meaningful and amazing event.


HFA SYMPOSIUM

BY ROCKY WILLIAMS

The 2024 Hemophilia Federation of America (HFA) Symposium was held in April this year in Indianapolis. Representing CHB, Wayne Cook, Dr. David Clark, and I exhibited and connected with the community.

One of the symposium's biggest highlights was the *On the Horizon* session on Saturday, April 13th, featuring our very own Dr. David Clark as a co-speaker alongside Dr. Steven Pipe. Dr. Clark delivered an insightful presentation on current products and gene therapy for bleeding disorders.






PARENTING SUPPORT: FROM NEWBORN TO EMPTY NEST

BY MARTA THOMAS

If you missed out on the recent *Parenting Support: Newborn to Empty Nest* session on April 3rd, let's catch you up on the action! It was a fantastic opportunity for parents and caregivers to come together, swap stories, and pick up some pearls of wisdom on navigating the challenges of raising kiddos with hemophilia B, some of whom are dealing with the condition themselves or are carriers.

This event, co-hosted by Cassandra Starks and Megan King, was all about creating a supportive space where folks could share their journeys and get some top-notch advice, and boy, did they deliver!

The evening kicked off with some good vibes, thanks to Cassandra and Megan's warm welcome and some tunes to set the mood. Then Carrie Koenig from Sanofi, our event sponsor, gave a shout out, setting the stage for a positive and uplifting session.

But the real fun began with an icebreaker led by Cassandra and Megan. It was a lively game of *Rock, Paper, Scissors, Shoot*. Yup, you heard that right! It had everyone in stitches and really got the ball rolling for some great conversations.

Now, let's talk about the main event: Ben Shuldiner, Superintendent of the Lansing School District, and a real pro when it comes to handling hemophilia B. His talk was like a masterclass in parenting with a side of empathy. Ben dished out some amazing tips on advocating for your child in school, making sure they get the support they need, and creating an environment where they can thrive. From kindergarten prep to college accommodations, he had information on it all. And you know what was the best part? The camaraderie among the parents. The session turned into a full-on chat fest where everyone shared their struggles, swapped advice, and lifted each other up. It was like one big support group, and it was beautiful to see.

As the evening wound down, folks stuck around to keep the conversation going. The connections made here were real, and I have a feeling they're going to last long after the event is over.

So, big shout out to Sanofi for making it all happen, and mark your calendars because we've got more sessions coming up on July 24th, Sept 18th, and Nov 6th. Can't wait to see you there!

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Comments:

"So grateful for the Coalition for this new, informative series. It is great to have a support system, such as this, to be able to turn to when I need answers!"

"The event was very helpful and informative. I particularly enjoyed the interaction between the families by sharing their experiences. I strongly believe that it's very positive for our community."

"Ben was a great speaker – very helpful, great energy and seems to genuinely care."

"I really enjoyed listening to Ben's advice for school age kids. His experience with hemophilia and as a professional was very helpful."

"My son isn't yet school aged, but I really appreciate hearing about others' experiences so we can plan and prepare appropriately!"

"I appreciate the connections with other parents. I always learn new things at these events!"

"This virtual event for parents was so good! I felt included, valued and grateful! CHB always takes such time and thought for us, and I will forever be thankful to "B" a part of this community! So many take aways for me to make sure I am doing my best for my hemo hero!"

LET'S PLAY NINE GOLF FUNDRAISER

BY HOPE WOODCOCK-ROSS









It was the perfect day for golfing on April 24th! We had 40 community members take to the greens at the beautiful Stevens Park Golf Course in Dallas in support of CHB to raise funds for The Coalition's *Let's Play Nine* golf outing.

Funds raised go to provide golf equipment and lessons for children with hemophilia so they can experience this fun, social activity, and its many health benefits and to our Patient Assistance Fund.

The event began with stretching exercises led by Kevin Harris. Then, Perry Parker took to the stage to demonstrate top-notch putting skills, setting the tone for the event. He held a clinic on putting that had us all engaged and improving our golf technique.

Once the golfing started, we had a great time on the greens. It was the perfect way to connect and compete. Huge congratulations to the first and second place teams. First place went to Kris, Kevin, Brian, and Craig. Second place went to Wayne III, Robb, Bill, and Keith. Also, congratulations go out to our *Longest Drive* winner Jordan.

A big thank you to all those who participated and made this day a tremendous success! Thank you also to our volunteers: Jennifer, Megan, and Deena! We also could not say thank you enough to our generous team sponsors!



















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APPLY TODAY: HEMOB.ORG/UPCOMING-EVENTS

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Women & Hemophilia B

Raffles!

Food vouchers for first 50 to register!

JOIN US AS WE AMPLIFY WOMEN'S VOICES IN HEMOPHILIA B CARE!

Let's get together to share strategies for women and girls to articulate their needs and advocate for comprehensive treatment.

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MATTHEW IS GOING THE DISTANCE!

BY SHELLY FISHER

Matthew took some time out from working on college applications to talk about his love for jazz music, the Coalition for Hemophilia B's *The Beats* music program in Nashville, TN, cross country, track, and his plans for the future. With a school year packed full of extracurricular pursuits that mean a lot to him, he was also carving out time to focus on the college admission process.

As a first-time attendee, this senior enjoyed traveling to a different part of the country to attend The Coalition for Hemophilia B's *The Beats* music program. While there, Matthew played both acoustic and electric guitar, and sang as well. His favorite part was at the end when everyone went up on the stage together. Matthew particularly loved playing as part of a group and thoroughly enjoyed the evenings after dinner, when he played music with other teens.

The program offered more than just performance opportunities. Matthew enjoyed learning about the music industry and audio production, broadening his understanding of the field. Perhaps most importantly, being with community members made him feel like part of a family and truly seen, especially since his hemophilia and regular life are often kept under wraps.

His attendance at *The Beats* music program was most likely no surprise to his family and friends. Matthew shared that he played guitar at performances with his school's jazz band throughout the school year, and he was excitedly anticipating some public performances that his teacher was looking into scheduling as well. In addition, he also plays with his local all-county jazz band, and he had the opportunity to play with jazz musicians from all around the area, as well as perform at nearby farmers' markets. Just this last spring, his love of music crossed over into song composition as well, and he was proud to share that he had performed some of his own songs at a farmers' market and the school's talent show.

As a member of the cross-country team, he wrapped up his last high school season just a few weeks prior to our visit. He shared that he loved the slower pace, less impact on his joints, and the warmer weather of the season. Matthew shared that his team was more competitive this year and also stepped up in their division rating.

Feeling as though it placed him in the minority, he shared that math, and calculus specifically, was his favorite subject. Why would this musician and track star choose that particular subject? "I'm pretty decent with numbers, and I can get through the homework faster than other classes." With a broad field and a lot of options to choose from, Matthew's love of math and chosen major, mechanical engineering, will serve him well!

When asked how his friends might describe him, this soon-to-be college freshman felt they would mention his love of music and call him the "therapist friend." With college on the minds of all of his friends, he told me that he encourages them "not to worry too much."

Diagnosed with hemophilia at birth, Matthew began self-infusing at about 9 years old and acknowledged his mom as a strong and consistent support throughout his life. He also mentioned Nurse Joseph Stanco and Dr. Suchitra S. Acharya at his local Hemophilia Treatment Center as always being accessible and supportive to his family.

His advice for anyone newly diagnosed? "Take your medication on schedule, live cautiously, avoid contact sports," and remember, "just because you have it doesn't mean you can't do things that are strenuous, it just takes more diligence. There are ways to develop underused muscles and types of physical therapy that can help hemophiliacs especially." Sounds like great advice for us all.

With a mantra like, "Done is better than perfect," this senior isn't obsessing over every little detail. He told me, "At the end of the day, I just call it done." As a senior keeping up with the requirements of the school year, continuing to do what he loves, and looking ahead to college, this phrase seems to be serving him well.

Best wishes to Matthew and his graduating class of 2024!

IN GOLF AND IN LIFE, COLLIN AND HIS PAW PAW ARE *"GOING FOR IT!"*

BY SHELLY FISHER

I am not sure who was more excited to see the other, Collin, or his Paw Paw, but one thing is for sure, it was obvious from the start that they were two peas in a pod! With Collin's mom, Lori, and Grandma Lynn (affectionately known as 'Honey') there as well, the guys were sure to get all the details correct!

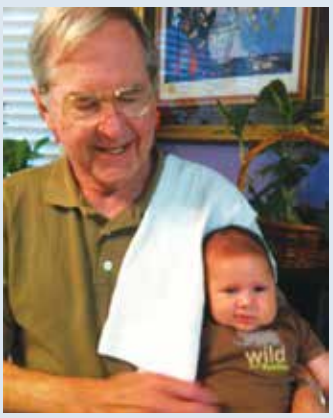
A fourth-grade homeschooling student, Collin was quick to name science and math as his favorite subjects. "I like to learn about robots. Me and my brother, Austin, want to own a zoo together." When asked why he wanted to own a zoo, Collin replied, "Well, my mom, my brother and I go to the zoo a lot and we just like it." Collin's mom, Lori, added that they had recently discovered that there are conservation engineers that use robots to work with animals and help with conservation and the whole family was pretty excited about it. How might the idea of owning a zoo and incorporating robots come about? It seemed that Collin had gotten a book for Christmas titled, "Everything Robots," by National Geographic, and the family has been researching conservation engineers ever since.

When he's not dreaming up the perfect family business, and pursuing his love of science and math, Collin participates in an online Bible Study Fellowship where he's met friends from all over the world, including England, Germany and South America.

While he enjoys tennis and soccer as well, it's clear that golf is Collin's game of choice, and he comes by it naturally as a third generation golfer. In addition to a great grandfather who was a golf professional, his Paw Paw's name is on the wall at Pinehurst. "I won the Donald Ross Junior Championship right after Christmas in 1961," Mr. Bennett said with a smile. It would be one of seven junior tournaments he would win at the age of 15. After hitting his first golf ball at the age of 2 1/2, he grew up playing golf with his dad, a golf professional in Florence, SC. "It's been in my blood a long time."

In addition to their love of golf, winning smiles and quick wit, Collin and his Paw Paw have also shared a few sand traps in their game - they both have hemophilia B. Collin was eight days old when they decided to test him due to his grandfather's diagnosis. The first injury he remembers occurred at the age of three when a disc golf frisbee hit him across the bridge of his nose. Fortunate to be part of a branch of St. Jude, Collin had staff waiting on him thanks to Operation Bleed. The result was three stitches, three injections of factor, a sucker, and a story. Unfortunately, his Paw Paw, Mr. Bennett wasn't so fortunate.

At the age of 24, he was getting ready to play the North and South Junior Championship at Pinehurst. He explained further, "I was playing a practice round on Pinehurst #2, came around to #5, par 4, uphill, dog leg left, and it started raining and lightning started



popping when we were on the green and there was a rain shelter right behind the green, so I go into the rain shelter." Mr. Bennett went on to tell how another golfer pulled his cart into the shelter and accidentally backed over him, injuring both of his knees and puncturing his chest with the umbrella. While he was home healing, he noticed a bump on his chest and went to the doctor to have it lanced. "In the fifties, that's what they did if you had a hematoma. No one really knew too much about hemophilia." The bump began to form again and started bleeding and the doctor told him there was something wrong with his blood. After a trip to Duke, and then Chapel Hill Hemophilia Hospital, Collin's Paw Paw was diagnosed with hemophilia B – 21% deficiency.

The advice that Collin and Mr. Bennett would offer someone who has just been diagnosed is, not surprisingly at all, very similar. Collin offered, "Don't be afraid of needles. You have to get them a lot when you have hemophilia." After hearing his advice, Mr. Bennett grinned and said, "Remember that Collin," to which everyone erupted into laughter and smiles. Collin also added that every time he plays a game he knows that he could get hurt and have to get factor "pretty fast," or just ice a hematoma like the one he got from getting kicked in the shin. Mr. Bennett shared that originally, Collin's parents didn't want him to play sports, but he wanted him to be a linebacker for Carolina, and even though they decided against football, he is tickled that he's playing golf and tennis." His advice for someone just diagnosed? "You know, you're possibly gonna get hurt, you should talk to your hematologist and ensure you have a good plan. If it's something you love, see if you can do it!" Collin smiled while listening to his Paw Paw and said that was his plan.

When asked if they had a favorite quote, or saying that they live their life by, Collin chose Philippians 4:13, "I can do all things through Christ who strengthens me." Mr. Bennett loved Collins' choice of verses and offered one of his favorite movie quotes from *Pretty Woman*, "Take the corners like you're on rails!"

It's definitely a family affair when it comes to supporting each other. When asked if anyone had been especially supportive of him, Collin pointed at his Paw Paw and

said, "This guy right here. When I am worried about the needles, he just says, 'It's gonna be alright!'" Mr. Bennett was supported through heart and hip surgery by his wife. "If it hadn't been for her I don't know if I would be talking to you now." Her knowledge of infusions now helps Collin as well. Collin's dad contributed as well by reading three separate books on hemophilia when Collin was born. Mr. Bennett credits Lori and her husband, Will, for being amazing parents to Collin and his brother.

Collin's mom Lori shared that she is thankful for organizations like the Coalition for Hemophilia B because "not only does it help us connect with other families, it helps us as a family have a place to get more information, feel supported, and fellowship. My mom just said the other day, that there's not a lot of people in their 70s with hemophilia, so it's good to make those connections." She added, "They've helped our family, and we get to meet other families going through some of the same hard things and who understand living with hemophilia B."

Collin enjoys the virtual events, and he was most looking forward to his grandparents flying in to meet them in Dallas for The Coalition for Hemophilia B's 2024 Annual Hybrid Symposium. It was his first in-person symposium, and it was a hit. Collin says that he enjoyed being with more people that have hemophilia B, and he says that he absolutely loved the Symposium!

B inspired!

Stories and artwork from teens in the Hemophilia B Community

SPRING 2024

IN THIS ISSUE:

MATTHEW IS GOING THE DISTANCE

IN GOLF AND IN LIFE, COLLIN AND HIS PAW PAW ARE "GOING FOR IT!"

WANTED: TEEN CONTENT CREATORS!

Calling all content creators! If you have a heart for tweens/teens and a drive for content creation, then we would love for you to volunteer your time and talents with us. The Coalition for Hemophilia B is currently accepting volunteers to collaborate on a new section of the newsletter just for those special 11–18 year olds in our community.

No experience required as we have a team ready to polish your brilliant ideas for publication. If you have ideas for topics, events, and new sections, let's work on this together – reach out to rockyw@hemob.org for your next steps!