

# KHF Hemosphere

## Camp Discovery

This year's summer camp was a wonderful experience for all kids and teens who participated. KHF's "Camp Discovery" takes place every year at Cedar Ridge Camp in Louisville, KY. A jam-packed activities schedule did not leave an opportunity for boredom or homesickness. Summer camp is such a meaningful and impactful program for every participating child and teen. Each and every one had a blast competing every day in the "Game of Games", playing "Capture the Flag," swimming, purchasing items in the "Canteen" with virtual dollars earned, and so much more. The final night event featured a Hawaiian/Polynesian theme reflected by food, beverages, decorations, and music. Every camp participant received a medal for completing the camp program and a certificate of achievement. We thank all our dedicated volunteers who made sure that lots of fun was had in a safe environment, our infirmiry nurses who took excellent care of our campers' medical needs, and our sponsors who helped make summer camp possible. They were Takeda, Kosair for Kids, WHAS Crusade for Children, CSL Behring, CVS Health, Novo Nordisk, Bayer HealthCare, Grifols, Pfizer, and Sanofi.





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



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

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

“Gene therapy is a complex biological ‘drug’ for which, despite 30 years of development, there are many unresolved questions, and the unknowns remain top of mind for clinicians and PwH alike. Evaluation of the risks and benefits of any new therapy requires the careful consideration of all the available information and a shared decision-making approach should be employed, explain the authors. “This is particularly important in the consideration of gene therapy, given the fact that AAV-mediated gene therapy is a one-time irreversible therapy. A fully informed decision must be ensured, and a robust shared decision-making approach is mandatory for these therapies.”

The review summarizes clinical trial data that supported the regulatory authorization of valoctocogene roxaparvovec in Europe to treat hemophilia A and etranacogene dezaparvovec-drlb in Europe and the [United States to treat hemophilia B](#). NOTE that since the publication of this paper, valoctocogene roxaparvovec also [received U.S. Food and Drug Administration approval](#) under the brand name ROCTAVIAN™.

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

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

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

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

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

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



## WE'RE IN THIS TOGETHER.

Friday 6:26 pm

Sharing stories by the campfire with friends

Isaac, living with hemophilia B

Not an actual patient

### Let's make today brilliant.

Takeda is here to support you throughout your journey and help you embrace life's possibilities. Our focus on factor treatments and educational programs, and our dedication to the bleeding disorders community, remain unchanged. And our commitment to patients, inspired by our vision for a bleed-free world is stronger than ever.

 [bleedingdisorders.com](https://bleedingdisorders.com) | 

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# Event News

## KHF Annual Meeting – Summer Family Event

The 2023 Annual Meeting-Summer Family Event took place on Saturday, September 16, at the Hyatt Regency Hotel in Louisville. After an initial exhibit period by industry, a full breakfast buffet was served during. The keynote speaker, Darlene Shelton, President and Founder of Danny's Dose Alliance, spoke about her family's life-changing diagnosis, and how they used it to "change rules" and protect the lives of millions of Americans with rare and chronic conditions requiring specialized emergency care. Breakout sessions were "8 Tips to Survive Adulting," presented by Judy Doyle, Novo Nordisk and "Nose Bleeds, Mouth Bleeds, Belly Bleeds, Oh My," presented by Missy Frey, RN, CVS Health. A lunch buffet followed during which announcements and reports were presented, elections were held, and a well-deserved drawing of door prizes commenced. The main prize, a Nintendo Switch, was won by Cameron Music from Lexington. Children who had participated in the day's children's program facilitated by Connie Thacker with the help of her friends received a very nice and "sweet" goodie bag. Many thanks to our volunteers and our sponsors. The volunteers were Pam Price, Laura Webb, Jennifer and Patrick Dunegan, Travis Price, Connie Thacker and friends, Jackson and Izzi Woods. Sponsors were Accredo, Bayer HealthCare, CSL Behring, Cottrill's, CVS Health, Genentech, HEMA Biologics, HPC, Novo Nordisk, Octapharma, Pfizer, Sanofi, Soleo Health, and Takeda.



## KHF Young Leader Awards

The Young Leader Awards are held annually at our Annual Meeting to teens and young adults, ages 17-25, who have demonstrated excellence in leadership in specific areas of service with KHF. This year, we recognized six amazing young leaders who have demonstrated their leadership skills during the past 12 months, August 2022 – July 2023. The awardees are: Taylor Currie, Louisville, for her service on the Camp Committee, as a Summer Camp Counselor, and for her photography skills; Quinn Jackson, Eminence, for serving as a Junior Counselor at Summer Camp after completing two years as a Junior Counselor in Training; Marissa Johns, Shepherdsville, for having been selected to serve as our representative in our national organization's National Youth Leadership Institute (NYLI); Sam Johnson, Louisville, for her service on the Camp Committee, as a Summer Camp and Family Camp Counselor, and as a peer advocate; Mason Stout, Somerset, for his service on the KHF Board of Directors and the Camp Committee, as a Summer Camp Counselor, and as a peer advocate; and Jackson Woods for serving as a Junior Counselor at Summer Camp after completing two years as a Junior Counselor in Training. These young leaders all received a very nice, inscribed glass award and a \$50 Amazon gift card.

## 2023 KHF Activities Calendar

**Nov-Dec.** Poinsettia Sale Fundraiser

**Dec 17** Year-End Community Event – German-American Club, Louisville



## 2023-2024 KHF Board and Officers

At our Annual Meeting – Summer Family Event in September, we welcomed Whitney Meadows to our Board of Directors.

Whitney and her family reside in Louisville. Whitney works for the US Senate as a Field Representative. We thanked and recognized our outgoing board member, Laura Webb, who served two three-year terms and held officer positions of president, vice president, and secretary. Officers elected for the 2023-2024 fiscal year are Eric Marcum, president; Andrew Hartmans, vice-president; and Aisha Irvin, Secretary.

## Fall Scholarship Awards

Post-secondary education scholarships were awarded for the fall semester to John Graham, Winchester; Alex Harmon, Bedford, and Ivy Webb, Louisville. The Herb Schlaughenhaupt, Jr. Memorial Scholarship was awarded to Ivy Webb. Ivy studies at Bellarmine University with a double major in Music Theory and Composition and Music Technology.

Ivy will graduate in May 2025 with 2 Bachelor's degrees in Music. Ivy's goal

is to teach music at the university level. The Theodore (Ted) L. Forcht Memorial Scholarship was awarded to John Graham who attends Centre College in Danville, Kentucky. He is a senior and expects to graduate with a Bachelor's degree in Finance and Economics with an emphasis on politics in May 2024. The Terry D. Turner Memorial Scholarship was awarded to Alex Harmon. Alex

attends Jefferson Community and Technical School. He will graduate with an

Associate in Arts degree in December. While pursuing his studies, Alex is employed by the Bedford Loan & Deposit Bank in loan processing. We congratulate the three recipients for their accomplishments and awards and wish them continued success with their studies.

The application submission deadline for the 2024 Spring semester is January 15, 2024.

You may obtain application form and guidelines by sending an email to [info@kyhemo.org](mailto:info@kyhemo.org) or calling 502-456-3233.



## Remember: KHF CARES



Kentucky Hemophilia Foundation continues to provide financial assistance to bleeding disorder families whose household income has decreased because of loss of job, lay off, furlough, reduced hours, or due to another type of emergency and as a result unable to pay a specific household bill. Requesting families must reside in Kentucky, and the person seeking assistance must either have a bleeding disorder or be the parent of a minor child with a bleeding disorder. Assistance is contingent on the availability of funds. Call 502-456-3233 or 800-582-CURE (2873) or send an email to [info@kyhemo.org](mailto:info@kyhemo.org) to make a request.

# More News

## Summer-Fall Donations

July 2023 – October 2023

We thank the following individuals and companies for their generous support!



### **Donor, \$3,200**

Mr. & Mrs. Terry Forcht  
for Poinsettia Fundraiser

### **Donor, \$1,700+**

Sanofi

### **Donor, \$750+**

CSL Behring

### **Donors, \$200+**

Bob Crawford  
for Walk  
Michael A. Gatton  
for Summer Camp  
Dianne Hartman  
for Walk

### **Donors, \$100+**

American Online Giving  
Dan Cummins Chevrolet Buick  
for Walk  
Cathy & Jewel Daugherty  
for Poinsettia Fundraiser  
Greg Fiscus  
Kroger Community Rewards  
Mona Lucky  
for Walk  
National Hemophilia Foundation

### **Donors, \$50 - \$90**

Kelly Hawkins  
for Walk  
Pamela Hazelip  
Linda Surratt  
Ursela Kamala  
for Walk

### **Donors, up to \$49**

Anonymous  
for Walk  
James Goalder  
Sharen Harmon  
for Walk  
Jim Huff  
Barbara Kinnaird  
for Walk  
Karen Lucky  
for Walk  
Marjorie Seely  
for Walk  
Nancy Ward



NATIONAL  
**BLEEDING DISORDERS**  
FOUNDATION  
*Formerly NHF*

## NBDF

National Hemophilia Foundation (NHF) is now National Bleeding Disorders Foundation (NBDF)

Please take note that our national organization recently changed its name and rebranded itself as National Bleeding Disorders Foundation (NBDF). This step was undertaken to show greater inclusivity. As an affiliated chapter of NBDF, KHF along with many others, welcomes and applauds this change.

# More News



## Kentucky Hemophilia Foundation Membership

July 1, 2023-June 30, 2024

We thank all members of the Kentucky Hemophilia Foundation who are supporting the current program year!

### Individual/Family Memberships, \$20

Travis Price  
Nita Wayne-Zehnder

### Patron Membership, \$50

Victoria Huff

Charles & Cheri Music

Laura & Glenn Webb

### Supporting Memberships, \$35

Judy Hayes  
in memory of Jason Hayes

### Sustaining Memberships, \$100

Megan Davidson  
Glen & Deborah Hitt  
Dr. David & Leslie Houvenagle

### Benefactor Memberships, \$250

Paula Bias  
Barbara W. Grayson

## In Memory

*Gone from our sight but never our memories; gone from our touch but never our hearts... May their memory be a blessing!*

### July 2023 – October 2023

Jeanne Hurt  
Frances K. Campbell  
Michael & Vanessa Creek  
Charles & Diane Tucker  
Margaret & Mark Vick

James Turner  
Nancy Ward

Terry Turner  
Nancy Ward

Mrs. Carol Nord  
John Nord, Sr.



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## Do The Five

*Follow these steps to prevent or reduce complications of bleeding disorders*

1. **Get an annual comprehensive checkup at a hemophilia treatment center.**
2. **Get vaccinated – Hepatitis A and B are preventable.**
3. **Treat bleeds early and adequately.**
4. **Exercise to protect your joints.**
5. **Get tested regularly for blood-borne infections.**

To find out more about the National Prevention Program developed by the National Hemophilia Foundation in collaboration with the Centers for Disease Control and Prevention (CDC), click on [www.hemophilia.org](http://www.hemophilia.org) or call toll-free 800-42-HANDI.



**Like us on Facebook and keep up-to-date on all KHF activities and events.**

KHF does not give medical advice or engage in the practice of medicine. KHF under no circumstances recommends particular treatments for specific individuals and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.



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