Spring/Summer 2022

KHF Hemosphere

2022 Family Day at the Zoo

This year was our 23rd annual Family Day at the Louisville Zoo, and it is still as popular as ever. One hundred fifty adults and children had a great time touring the zoo's many exciting animal exhibits, including an interactive petting zoo and a chance to ride a camel.

Our guests gathered at the spacious Oasis tent for a delicious picnic lunch, complete with door prizes for the adults and carnival games for the kids, and an opportunity to chat with exhibitors for cutting edge information regarding their products and services. After the welcome period of respite in the Oasis tent, many of our guests ventured back out into the zoo. Although it was a very hot day, everyone seemed to be in good spirits and happy to interact and visit with other community members.

We thank our exhibitors for their support of this family event. They are Biomatrix, CSL Behring, CVS Health, Grifols, Novo Nordisk, Octapharma, Pfizer, and Sanofi. We also thank our volunteers who helped ensure the success of this important and enjoyable event. They are Connie Thacker and granddaughter Kayden; our board president, Laura Webb; our board members Patrick and Jennifer Dunegan and Travis Price, our intern Samantha Johnson and her fiancé Nick Mazat, Brandy Fox, and Milton Kamala.

Special News

New Paper Highlights Integrated Care Model and the National HTC Network

For more than 40 years, the hemophilia treatment center (HTC), the U.S. HTC Network (USHTCN), and its model of integrated, patient-centered care has been essential in helping people with hemophilia (PWH) achieve optimal health outcomes.

Authors of a new paper posit that while the value of these specialized centers is well understood by central stakeholders, purchasers of health insurance and the broader medical community may not be privy to the full breadth and scope of the USHTCN, its intricate and deliberate structure, and its adeptness at meeting the physical,psychosocial, emotional needs of its patients.

The paper, “Integrated Hemophilia Patient Care via a National Network of Care Centers in the United States: A Model for Rare Coagulation Disorders,” was published in the Journal of Blood Medicine (JBM). It lays out the key components of the integrated care model, the structural underpinnings of the USHTCN, and the myriad of benefits to patients.

The authors describe HTCs functioning as highly coordinated, multidisciplinary teams within an evolving national network, uniquely positioned to deliver holistic care to an expanding patient population. Over time, this patient population has grown to encompass von Willebrand disease, rare factor deficiencies, and underserved groups including women and girls with inheritable bleeding disorders.

The authors define the fundamental staffing components at the core of every federally funded HTC, including a hematologist, registered nurse, physical therapist, and social worker, plus key extended multidisciplinary team members such as genetic counselors, orthopedic surgeons, oral surgeons/dentists, nutritionists, and other providers.

The paper also provides an accounting of the critical services and dynamic coordinating capacity of HTCs within a regional and national framework. The benefits of such an integrated system are vast, and include timely specialty laboratory services, coordinated pharmacy services, robust data collection, adherence to clinical guidelines, standards of care and consistent provider education/training opportunities. These and other aspects of integrated care have a profound, real-world impact on patients. “These services ensure that PWH and other congenital coagulation disorders have access to highly specialized care to reduce morbidity and enhance wellness, promote a longer lifespan, and improve patient/family functioning; while at the same time reducing avoidable emergency room visits, hospitalizations, and overall costs.”

The positive impact of these and other services on patients at HTCs are captured via periodic national patient satisfaction surveys with the topline results included in this paper.

The authors also explain the current funding mechanisms that support HTCs, and theassociated challenges, including fair and optimal reimbursement for services and patient access to the USHTCN.

Readers of this article will walk away with a strong sense of HTCs and the USHTCN as the cornerstone of optimal care for patients with inheritable bleeding disorders.

“Herein, we have described what HTCs do and how they do it through a team-based, multidisciplinary approach that incorporates the affected person in shared decision making. We have shown that PWH/caregivers are highly

satisfied and have presented evidence for why this model of care has been and will remain the gold standard in the United States," conclude the authors.

"This article can serve as a reference document for all stakeholders in the care of PWH and other rare coagulation disorders Taking all the benefits into account, we believe that purchasers of health care will conclude, as we do, that HTCs provide the highestquality care for their beneficiaries, delivering optimal health outcomes at the lowest total cost of care."

The paper, which was published online by JBM on October 21, 2021, is open access

Valentino LA, Baker JR, Butler R, Escobar M, Frick N, Karp S, Koulianos K, Lattimore S,Nugent D, Pugliese JN, Recht M, Reding MT, Rice M, Thibodeaux CB, Skinner M.Integrated Hemophilia Patient Care via a National Network of Care Centers in the United States: A Model for Rare Coagulation Disorders. J Blood Med. 2021 Oct 21; 12:897 911. doi: 10.2147/JBM.S325031. PMID: 34707421; PMCID: PMC8544265.

NHLBI Grant Supports Scientific Research to Enhance Hemophilia a Gene Therapies

A group of investigators are embarking on a new research program designed to unpack some of the outstanding fundamental questions associated with current approaches to gene therapy for hemophilia A. While multiple gene therapies are currently in various stages of preclinical or clinical studies there remain concerns relevant to the biological nuances affecting long term safety and efficacy.

The new program, which is being supported by a $12 million grant from the National Heart Lung and Blood Institute (NHLBI), represents a multi-institutional effort to ultimately help improve these therapies. It will be led by Roland Herzog, PhD, at the Indiana University (IU) School of Medicine.

Herzog is the Director of IU's Gene and Cell Therapy Program. He was also a recipient of NHF's Career Development Award from 2000-2003, for his funded project "Immunology of Liver-Derived Expression of Factor IX from AAV Vectors."

"Several companies have taken this forward into clinical trials, and in some of these trials, the patients initially looked like they were cured," said Herzog, who is the Riley Children's Foundation Professor of Immunology. "But what they all have in common is that they need to deliver a lot of the virus in order to get the desired results, and over time clotting factor levels started to decline. So, it's clear that we need to further study the biology of this phenomenon."

According to an IU School of Medicine press release this program will include three major projects, with investigators conducting molecular-level analysis of key components in liver-directed gene therapy, including human liver cells and factor VIII (FVIII) viral vectors. Herzog and his colleagues hope to garner new insights that can ultimately lead to lower levels of cellular toxicity and "improved longevity of FVIII production" in individuals who are treated with gene therapy for hemophilia A

"This is an incredibly significant and urgent medical question, and it requires the synergy of multiple groups with different expertise to come together and solve a problem that they wouldn't be able to solve on their own," said Herzog. "My hope is that our studies will help the field as a whole move toward curing hemophilia A."

Source: Indiana University School of Medicine news release dated March 8, 2022

Event News

Play a Round for a Cure Golf Scramble

KHFs' 32nd Play a Round for a Cure golf scramble went off without a hitch at Glen Oaks Golf and Country Club in Prospect. Eighteen foursomes teed off on a sunny June morning for a fun-filled day of golf. Box lunches, snack, and libations sustained our golfers on a hot and sunny day and on a challenging golf course. Our golfers persevered through eighteen holes just in time for dinner and awards. After a welcome cooling off period in the clubhouse and a light dinner, we congratulated and awarded prizes to the following winners: Longest/ Straightest Drive, "Cadillac" Sweeney; Longest Drive, Nick Miller; and four closest-to-the-pin prizes went to Mark Britt, Vince Poma, Reid Thacker, and Brandon Mullins. The winning teams were in 3rd place, "Trevor Black's Friends; in 2nd place, Aaron Lopez and Friends; and in 1st place, CSL Behring. Half of the $600 Ball Drop was won by George Fisher, and half of the $390 50/50 raffle was won by Jason Newton. An array of attractive silent auction items also ended up with happy winners.

We thank all companies and individuals who supported this long-standing fundraiser with their sponsorships, monetary and in-kind contributions. We also thank our golf committee, led by Chair, William Black, and our day of event volunteers who planned and held an exciting and successful event. Sponsors and contributors were: Gold level, Kosair Charities, Bayer HealthCare, and CSL Behring; Bronze level, HEMA Biologics, Octapharma, and Republic Bank and Trust Company; Team Plus level, BioMarin and LTC (R) John and Mrs. Patricia Tharp, in Memory of Gary F. Bandy; Team sponsor level, Kosair Golf Club and Marwood Manufacturing; Business Tee sponsors, Novo Nordisk, Marwood Manufacturing, and Medexus. Representatives from Marwood Manufacturing held a raffle at their designated tee box for a great basket of spirits and kindly donated the proceeds of $205 to KHF. All proceeds from this fundraiser help KHF fund programs and services for Kentucky's bleeding disorders community whom we have been serving for more than 60 years.

2022 KHF Activities Calendar

All activities listed are in person

July 24-28 Camp Discovery for Children and Teens, Cedar Ridge Camp, Louisville, KY

Sept. 17 Summer Family Event, Hyatt Regency Louisville, Louisville, KY

Sept. 30 – Oct. 2 Family Camp, Cedar Ridge Camp, Louisville, KY

Oct. 22 9th Annual Unite Walk, E. P. "Tom" Sawyer State Park, Louisville, KY

Dec. 4 Year-End Family Event, Holy Trinity Clifton Campus, Louisville, KY

Event News

Washington Days

Washington Days is the National Hemophilia Foundation's (NHF) annual advocacy event in Washington, DC. For the past two years, this large advocacy effort has been virtual. Several hundred bleeding disorders representatives from across the country participate each year. In 2022, the primary focus was seeking support for the HELP Copays Act (HR 5801), which is a bipartisan bill that was introduced by Reps. McEachin (D-VA) and Davis (R-IL). The two main points of the bill are to 1) clarify the Affordable Care Act's definition for cost sharing to ensure that payments "by or on behalf of" patients count. 2) to close an essential health benefit (EHB) loophole, making any covered item or service part of the EHB package, so that all cost sharing counts. We thank our Kentucky advocates who participated in this important national advocacy event. They were Laura Webb, Isaac Webb, Eric Marcum, Ursela Kamala and Lisa Raterman from the Tri-State Bleeding Disorder Foundation.

KHF Advocacy Day

Peer advocacy is an important component of KHF activities. We held our ninth consecutive Kentucky Advocacy Day to which our Kentucky bleeding disorders community was invited to give bleeding disorders a voice and face during meetings with Kentucky legislators. Our goal is to 1) meet with key state legislators and educate them about hemophilia, von Willebrand disease, and similar bleeding disorders, as well as related issues; 2) express concerns regarding availability, affordability, and access to care; 3) seek support for legislation that will benefit their community and others with chronic, rare, and high-cost health conditions. This year, we were tasked with seeking support for a "Step Therapy Reform Bill." "Step Therapy" is also referred to as "Fail First," which is a practice by some health insurers and pharmacy benefit managers (PBM) to require patients to try one or more medications that they select (typically cheaper drugs) before they will cover a higher cost medication prescribed by the patient's physician. SB 140, this year's "Step Therapy Reform Bill," was introduced by state Senator Max Wise and includes several clarifications and patient safeguards ensuring that patients receive the most appropriate medication at the right time. This bill moved fairly quickly and with only minor modifications through the state House and Senate and was signed into law by Governor Andy Beshear this spring. KHF and our dedicated group of peer advocates were eager to ask for support for this important bill during our 2022 Kentucky Advocacy Day activities. We were delighted and pleased when it became law in the Commonwealth benefitting our bleeding disorders community and many other individuals with chronic, rare, and high-cost health conditions. We thank Jim Romano of Care and Cure Partners for his assistance with a remote training session, setting up virtual legislator meetings, and facilitating the meetings via Zoom. We thank our peer advocates for their enthusiasm and commitment to advocating for themselves and others affected by bleeding disorders. They were William Black, Sara Ceresa, Ursela Kamala, Eric Marcum, Lisa Raterman, Isaac Webb, and Laura Webb. We thank Tri-State Bleeding Disorder Foundation for partnering with us in this vital endeavor. We also thank our sponsors for supporting this and other advocacy activities throughout the year. They were Takeda, Genentech, PhRMA, CSL Behring, Sanofi, and Pfizer.

More News

Kentucky Hemophilia Foundation Membership

July 1, 2021 – June 30, 2022

We thank all of the members of KHF who are supporting the current program year!

Individual/Family Memberships, $20+
Michael & Cathy Johnson
Nita Wayne-Zehnder

Supporting Memberships, $35+
Judy Hayes
in memory of Jason Hayes
John L. Silletto

Patron Membership, $50+
Mary E. Marasa

Sustaining Memberships, $100+
Arthur Hackman
John & Leah Graham
Barbara W. Grayson
D. Spalding Grayson
Dr. David & Leslie Houvenagle

Benefactor Memberships, $250+
Glen & Deborah Hitt
Ruth Ann LeVay
Eric & Venus Marcum
Laura & Glenn Webb

Champion/Corporate Membership, $500+
LTC (R) John & Patricia Tharp

2022 Spring – Summer Donations

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

Donor, $2,500
Pfizer

Donor, $1,500
Optum Rx

Donors, $750
CSL Behring
Novo Nordisk

Donor, $400+
Dianne Hardman
for KHF Unite Walk

Donors, $100+
Kroger Community Rewards

Donors, $50 - $99
Curtis & Winfred Jacobs
for Easter Lily Fundraiser

Donors, Up to $49
Amazon Smiles
Dolores T. Davis
for Easter Lily Fundraiser
Jenifer Schultz
for Easter Lily Fundraiser
Richard Sloan

In Memory

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

April – May 2022

Donald S. Grayson
Connie Adkins
Ann & Bill Borders
Jack & Delene Dentinger
Libby & Charlie Haydon
Rosemary Johnson-Dean
Sally (Sarah) K. Newcomb
Lois Odaniel & Joan
Pat & Teddy Privitt
Ronald McDonald House Charities of Kentuckiana
Angie Tucker Vann

Robert B. Johnson
Rosemary Johnson-Dean

New Data Analysis Sheds Light on Bleeding Patterns in Young VWD Patients

Historically, data on infants and toddlers (ITs) with von Willebrand disease (VWD), particularly relevant to bleeding patterns, has been lacking. To address this absence of data, a team of researchers from the U.S. Hemophilia TreatmentCenter Network (USHTCN) and the Centers for Disease Control and Preventionconducted a retrospective analysis of HTC patients with VWD who are less than two years of age. The data were obtained through the USHTCN. The results werepublished in the journal Blood Advances.

A total of 105 VWD patients two years of age or less were ultimately included in the analysis: of those, 63% were type I VWD, 28% were type II VWD, and 9% were type III VWD. Investigators focused primarily on birth characteristics, bleeding episodes, and complications.

A review of birth and delivery data showed that 86% were delivered at full term,82% were of normal weight, and 89% were considered to be of normal length. 63% of the births were done vaginally, while elective cesarean sections were utilized more often with mothers who were known carriers of VWD.

An examination of diagnosis data showed that patients with type 2 VWD were identified sooner, on average, than types 1 and 3. Patients had a mean age at diagnosis of seven months, with little variation by sex. A family history of VWD was also associated with an earlier diagnosis, occurring approximately four monthsearlier than in those without such a history. In all, family history of a bleeding disorder prompted diagnostic testing for 68% of this population.

The majority of the patients (70%) experienced a bleeding event, with 68% of those having their initial bleed in the first year of life. Initial bleeds most often occurred in the oral mucosa (mucous membrane lining the inside of the mouth), with 32% of patients experiencing this symptom. The second and third most common bleeds were circumcision-related (12%) and intracranial/extracranial bleeding (10%), respectively. Approximately 5% of patients suffered an intracranial hemorrhage, though none was associated with delivery at birth.

Treatment with bleeding disorder therapies was utilized in approximately 64% of the patients in the study, with nearly half (47%) of these receiving plasma-derived von Willebrand factor VIII concentrates. Aminocaproic acid was used in 32% of patients, while 14% received intravenous or intranasal desmopressin.

The authors highlight the central role HTCs play in mitigating risk and ensuring the best possible outcomes for these patients.

“Other studies have recommended a multidisciplinary care approach to provide early diagnosis and optimal care for this population. Specialized HTCs are uniquely positioned to offer such multidisciplinary care, including genetic counselors throughout the prepartum period who work to increase expectant mothers’ understanding of the risks associated with having a child with VWD, and adult and pediatric hematologists, obstetrician-gynecologists, genetic counselors, nurses, and social workers throughout the pre- and postpartum period who seek to optimize outcomes and disease management,” conclude the authors.

Source: Hematology Advisor, October 4, 2021

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 or call toll-free 800-42-HANDI.

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

KOSAIR CHARITIES

WHAS CRUSADE FOR CHILDREN

KENTUCKY HEMOPHILIA FOUNDATION
1850 Taylor Avenue #2
Louisville, KY 40213-1594

Non Profit Org.
U.S. Postage
PAID
Louisville, KY
Permit No. 883

WHAT'S NEXT? **YOU** DECIDE.

At Genentech, we're committed to creating programs for you, with you. From a web series focused on finding the *magic* in life, to a tournament for gamers, to workshops designed to help you think well, do well, and be well, we're here to help you take on what comes next.

VISIT **GENENTECHHEMOPHILIA.COM** TO SEE HOW WE'RE CREATING WHAT'S NEXT, TOGETHER.

GENENTECH IN HEMOPHILIA | Genentech *A Member of the Roche Group*

2022 KHF Activities Calendar

July 24-28	Camp Discovery for Children and Teens, Cedar Ridge Camp, Louisville, KY
Sept. 17	Summer Family Event, Hyatt Regency Louisville, Louisville, KY
Sept. 30 – Oct. 2	Family Camp, Cedar Ridge Camp, Louisville, KY
Oct. 22	9th Annual Unite Walk, E. P. "Tom" Sawyer State Park, Louisville, KY
Dec. 4	Year-End Family Event, Holy Trinity Clifton Campus, Louisville, KY

All activities listed are in person

Before you know it....

The KHF Unite Walk may not be until October 22, but now is the perfect time to put together your team, plan your strategy, and start fund-raising. You know the winning teams get great prizes, and that the money you raise goes to a great cause.

Look for your 2022 Walk brochure coming in the mail soon. Show your support for the bleeding community and sign up ASAP. For any questions, contact KHF. ♦ (502) 456-3233 (800) 582-CURE (2873) ♦ FAX (502) 456-3234 www.kyhemo.org ♦ info@kyhemo.org

See you there!