

The News Infusion

The Official Newsletter of Bleeding Disorders Association of South Carolina

VOLUME 11, ISSUE 1

WINTER 2022

March National Bleeding Disorders Awareness Month

RED TIE CAMPAIGN

FOR ALL BLEEDING DISORDERS

Care

Compassioned ♥ Advocates
Resilient ♥ Empowered



BDASC

APRIL 17
2022

WORLD
HEMOPHILIA
DAY
#WHD2022



STEP

FOR BLEEDING DISORDERS



Featured Articles

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HELLO: June Annual Meeting and Conference, page 8

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BDASC MISSION: To raise awareness for and advocate on behalf of persons with bleeding disorders and their families; provide education and supportive services; and promote ongoing research to improve the quality of life for those affected. **BDASC VISION:** The Bleeding Disorders Association of South Carolina is to be recognized as a leading organization providing valued services; as ambassadors of public outreach to enlighten and foster an understanding of what matters most to those affected by hemophilia and bleeding disorders; and to be our community's first choice in partnership to achieve their highest potential through empowerment, connection to their community and being part of the solutions that affect them the most, until a cure is achieved.

2022 Calendar of Events

Please visit our website calendar page link to see the most current information on scheduled events <https://bda-sc.org/events/>

February 19 — CARE: Ladies Night Out (Fort Mill)

February 26 — Men's Support Group (Greenville)

February 28 — Rare Disease Day (Virtual)

March 1-2 — NHF Washington Days (Virtual)

March 10 — Men's Program Meeting (Virtual)

March 8-9 — BDASC's Advocacy Days (Columbia)

March 25-27 — BDASC's Women's Bleeding Disorders Conference (Greenville)

April 7-9 — State of the Science Research Group to Washington, DC

April 15 — World Hemophilia Day Celebration (Greenville)

April 16 — World Hemophilia Day Celebration (Columbia)

A Chapter of the National Hemophilia Foundation

A Chapter Member of the Hemophilia Federation of America

A Registered Greenville County Charitable Organization

Member of the Greenville Chamber of Commerce

A Proud Member of the South Carolina Associations of Non Profit Organizations - Together SC

April 17 — World Hemophilia Day (Recognized)

April 20-23 — HFA Symposium (Houston)

April 29 — BDASC Academic Scholarship Program Deadline

April 29 — CARE: Ladies Night Out (Columbia)

May 10 — Bleeding Disorders Advisory Committee (Industry) Meeting

May 12 — Men's Program Meeting (Virtual)

May 14 — STEP for Bleeding Disorders (Saluda Shoals Park, Irmo)

May 28 — YES: Young Families Empowerment & Support (Columbia)

June 10-11 — BDASC Annual Meeting & HELLO Conference (Greenville)

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About This Publication

The News Infusion's mission is to provide communication, connections, education and advocacy awareness. It is published quarterly by the Bleeding Disorders Association of South Carolina as an informational service to its members, friends, and affiliates of South Carolina's bleeding disorders community. *The News Infusion* does not endorse any provider, company or product, and further recommends that its readers always consult with their physicians and health care providers. *The News Infusion* is for educational and communication purposes only and makes no claim to its accuracy. *The News Infusion* is endorsed by the South Carolina Hemophilia Treatment Center and the National Hemophilia Foundation. We encourage your feedback and communications as a forum for exchange of information, ideas, and opinions. We welcome your letters, articles, questions and pictures. All articles may be published or distributed in print, electronic, online or in other forms. All submissions will be verified and subject to editing. Please send to the editor-in-chief at: sue.martin@bda-sc.org. Mail to: 25 Woods Lake Road, Suite #300, Greenville, SC 29607, Attention: Editor-in-Chief, The News Infusion. We look forward to hearing from you soon!

Lead Article

Inaugural Women's Bleeding Disorders Retreat & Conference

MARCH 25-27, 2022

SpringHill Suites Greenville Downtown

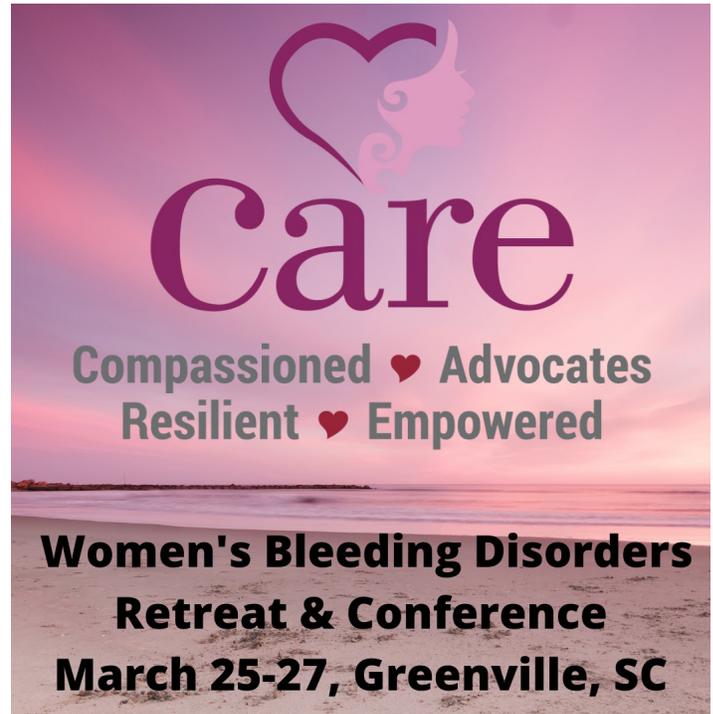
**200 E. Washington Street
Greenville, SC 29601**

Deadline to register for rooms—March 2

We are so excited to host the first conference intended for women who are affected by hemophilia, von Willebrand Disease (VWD), or other rare bleeding and platelet disorders. This includes: women who are carriers of genetic bleeding disorders, are caretakers of loved ones with bleeding disorders, and women with daughters who are obligate carriers. Join us for conversations and education providing support, friendship, and understanding, while learning how to advocate for women's health care treatment needs. Come learn and understand the new guidelines for VWD management, treatment, and diagnosis. There is a great deal of movement in understanding and supporting women who bleed, and we invite you to share your voice, your experiences and concerns while helping address the needs of our "Women Who Bleed." This weekend is for adult women with no onsite childcare offered. Financial stipend assistance for childcare may be available for those who request this assistance to attend. We are planning a wonderful weekend for you! Limited space is available so don't wait! Register today. For questions, contact the office.

View our [agenda](#) of fabulous presentations just for women!

Women Who Bleed (*Sponsored by Bayer*) This session empowers women to know the meaning of their hemophilia and bleeding disorders carrier status and understand bleeding and treatment options. The program focuses on going beyond that carrier status, getting tested, knowing that



women may need to treat depending on their factor levels.

Mind, Body, Heart, Connection: Women's Mental Health (*Sponsored by HFA*) This session concentrates on taking a holistic approach and how all these functions work together supporting attendees to create a more balanced life and empowers attendees to learn to build coping strategies and take control of their health, body, and mind for a healthy lifestyle.

Reproductive Health, Menstruation, and Menopause (*Sponsored by NHF*) Reproductive health, menstruation, and menopause are vital topics in the lives of girls, women, and those who have the potential to menstruate. Yet, our conversations about these within the inherited blood disorder community have been sparse.

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It's time to change. This workshop will provide a deeper dive into physiological components of these, highlight implications for bleeding disorders, and provide essential skills and resources to the participants.

Carrier Guilt (*Sponsored by HFA*) Join Anna Bell as she facilitates a rap session among attendees about the feelings women face and their concerns about possibly passing along a bleeding disorder. This session is an open conversation for women to share, connect, and support one another on being a carrier of a genetic bleeding disorder.

We all have "Common Factors" (*Sponsored by CSL Behring*) Hear from our Common Factor Advocate about living with a bleeding disorder, or being a caregiver of someone who does, and how to "make yourself a priority." This session will offer insight into different ways you can learn too how to "laugh through the stress."

VWD Overview (*Sponsored by Grifols*) This interactive program will provide a history of VWD, its diagnosis, laboratory testing and classification of subtypes, as well as the current trends in the management of VWD, including the consensus data on reproductive issues.

VWD Guidelines: What They Mean for You – New VWD Guidelines and Management (*Sponsored by NHF*) In 2021, the new VWD guidelines on diagnosis and treatment were published. In this workshop, participants will learn more about the innovative international development and the most important changes. But even more so, participants will learn what these guidelines mean for them and how to use these as a foundation for shared decision making for their own best care.

Women Matter in Bleeding Disorders (*Sponsored by Takeda*) Women with bleeding disorders face unique challenges that are often unrecognized or underappreciated. Understanding some of the special challenges faced by women with bleeding disorders and



identify options for emotional support within the community.

Activities: Friday night fun for women to connect! Enjoy *Ribbons and Burlap* for an Easter or Spring Art Project – www.facebook.com/burlapandribbons/. Saturday after the conference session we will relax with Hope Brooks for a little Yoga time, dinner, and a night on the town with friends. We are grateful to our sponsors for their support of this important conference to address women's health equity.

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for all bleeding disorders



Dedicated and Committed to the Bleeding Disorders Community

The Bleeding Disorders Association of South Carolina Board of Directors is responsible for the governance of the organization and works closely with the executive director and staff. The team meets monthly to discuss the direction of programs, services, and support for the membership. With some members ending their terms this June, new board positions will be available. We are actively searching for committed members to serve in this leadership role. If you or anyone you know, including someone outside the bleeding disorder community, would be a great addition to our team, please send them our way! Additional information is on our website [About Us](#) page including the new board member application, or you can contact the office at info@bda-sc.org or 864-350-9941. We would love to share the visions we have for our future with new members. Dr. Joey Krakowiak shares his views of his time serving the community below.

I joined the BDASC Board of Directors in late 2020 after being an active member since 2019 after my son, Thomas, was born with severe Hemophilia A. It has been a great experience working with a great group of people who all care deeply



about our community. My favorite aspect of being a board member has been how much I have learned about the bleeding disorders community in such a short time. I have learned so much about the unique challenges that we all face and how

we can strategize to meet those challenges and best support our bleeding disorders family in South Carolina. I'm a busy physician with two small children among other responsibilities, yet the expectations and responsibilities of being a board member are still doable because we



work as a team and share the work. Being a board member so far has been very fulfilling and I look forward to continuing our work. And I encourage you to join me and Sue and the rest of the board too! If you are interested in gaining experience with a great nonprofit, or you perhaps have a need or desire for community service, or you would like to know more about how you can help set up the bleeding disorders community for success in

the future, then I would highly suggest you reach out to any of us on the board or Sue for more information.

— Joey Krakowiak

Apply For Our Academic Scholarships

Every year, the Bleeding Disorders Association of South Carolina sets aside general funds to support higher education scholarships. To be considered for a scholarship, an applicant must meet the following criteria: must be a resident of South Carolina; must be enrolled or accepted at an accredited educational institution, to include accredited colleges, universities, and technical or vocational schools; must have a bleeding disorder; or be a dependent child of a person with a bleeding disorder; be a sibling of a person with a bleeding disorder; be a parent of a dependent child with a bleeding disorder; or be a spouse of someone with a bleeding disorder. Applications must be completed and received before the deadline dates. The Chapter will determine each year how many scholarships to award depending on funds received within each year and the number of qualified applications. The application and all supporting materials must be postmarked

by **April 29, 2022**, in order to be considered. Incomplete applications will not be evaluated. Please plan to send early so we can receive all applications by **April 29, 2022**, for quick consideration. Be sure all recommendation letters are received by the deadline date of **April 29, 2022**, to complete your application if coming from the individual making the recommendation on your behalf. For questions and the 2022 application, visit our website at www.bda-sc.org/academic-scholarship-program/program or reach out to us at 864-350-9941.

We are honored to have an additional scholarship to award this year through the generous donations in honor and in memorial of **Roy Clifford Sperry**, father of Shawn Sperry, past board member, whose family are longtime members of HSC / BDASC. We wish to send our condolences to the Sperry family and thank them for the opportunity for additional community support.



Executive's Welcome

By Sue Martin

A New Year and a New Us!

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Happy New Year to all our members, their families, and our friends! It is hard to believe we are heading into Spring, and I'll personally be happy to enjoy warmer weather. We haven't stopped working hard however throughout the winter: planning out the 2022 calendar, strategizing at our annual board summit, hosting virtual and live events for community connections, and analyzing our [member feedback survey](#). We are still waiting for our final IRS designation with our new name (they tell me they are very busy); however the [new website](#), email addresses, home office and fax number, and a NEW LOCATION for the organization, all are very exciting. The new office will be better suited to host meetings and visits with members and their families. I am even considering virtual office hours where you can join me and community members through Zoom! Once the move is complete and we are all adjusted, we'll have an open house in celebration of all our transitional adjustments. We are grateful to our members who we serve and with our more inclusive name and a bigger office setting, we can anticipate our membership will continue to grow. Funding will be critical this year as many new adjustments in our revenue stream continue. We value our partnerships with our industry community and understand that all companies are evolving with the new changes and challenging times. I hope to attract new

volunteers to the office as I can really use your help for events and office work, planning, and helping with the fundraisers and conferences. If you need somewhere to spend your time, call me! How about some "lunch and learns" in the new board room? With many new potentials, my visions continue to develop with endless possibilities.

I'll keep it short and ask that you take the time to enjoy reading what we have been up to since we last met. Please continue to reach out if you need anything and if we can be of assistance. *PLEASE* join me at our events as much as possible so I can attract the best in education and support services for the community. I do understand we are still in a pandemic and your safety is always of the utmost importance. We will do our best to keep you safe as we move forward to live events in 2022 and will follow all COVID 19 protocols. Continue to join us online too! Our virtual technology will not stop once COVID is gone (someday). I hope you will join us during the National Bleeding Disorders Month of Awareness in March, secure a proclamation for us, make a donation to the [Red Tie Campaign](#), and connect at the events we have planned in honor of our special month of recognition! The [STEP for Bleeding Disorders](#) walk is May 14 and you can read more about it on page 13. I will be calling you for volunteer help and ask that we all make an effort to raise funds to support the organization in all we do. And don't forget about our June Annual Meeting and HELLO Conference! It is going to be great! Read more on page 8.

I wish to also thank Greg for our time together at BDASC and wish him all the best in the future. I hope your Spring is beautiful, and in the spirit of the community in South Carolina, I wish you all the very best!

Warmly,
Sue

Community Engagements

SAVE THE DATE! **We Are Over Halfway to HELLO!**



June 10-11, 2022

Friday 5:00 p.m. to Saturday 8:00 p.m.

**VENUE: Embassy Suites by Hilton Greenville
Golf Resort & Conference Center**

670 Verdae Blvd, Greenville, SC 29607

**Childcare Onsite Services Will Be Provided by
[Corporate Kids Events](#)**

There may still be a chill in the air, but planning for the June 10-11, 2022, Annual Meeting and HELLO Conference is well underway. Scheduled at the Embassy Suites by Hilton Golf Resort & Conference Center in Greenville, this will be BDASC's 49th Annual Meeting. It provides the South Carolina bleeding disorders community the opportunity to come together, network, learn, and enjoy the empowerment of education and support. As always, we will be holding the presentation of the Chapter's Annual Awards on Friday night, including awarding of college scholarships. We will also be introducing our new board members and celebrating those who have served and are now stepping down.

While we are still organizing the breakout sessions, we have secured our keynote speaker for this year, Kevin Mills, Ph.D., Chief Scientific Officer, National Hemophilia Foundation. Dr. Mills joined the National Hemophilia Foundation as chief scientific officer. A graduate of the University of Colorado Boulder, MIT, and Harvard

Medical School, he is responsible for overseeing NHF's research strategy, including developing and expanding the foundation's research capabilities, and setting research goals. We have been working side by side with NHF on advancing research and are so honored Dr. Mills has accepted our invitation to meet the South Carolina Community and share his visions for research advancing patient treatment. Dr. Mills brings more than 20 years of experience in biotechnology, hematology, and oncology to the role. In 2005, Dr. Mills joined the faculty at the Jackson Laboratory in Bar Harbor, Maine, and led a laboratory focused on the biology of DNA repair in hematology and oncology. In 2012, he co-founded Cyteir Therapeutics, a biotechnology company developing and commercializing new therapies targeting DNA repair pathways. As Cyteir's chief scientific officer, he led the team that brought a first-in-class RAD51 inhibitor into clinical trials. Dr. Mills subsequently joined Civetta Therapeutics as chief scientific officer.

We are planning a teen track that will run concurrently with our adult programming, so there will be something for everyone to learn. The HELLO Conference allows attendees to enjoy a learning environment with educational presentations and breakout sessions. We learned in 2020 and again in 2021, that our online ZOOM platform helped us reach a broader audience, including those who were unable to join due to distance, health, or age, giving them the opportunity to gather together with us in unity. We will work to provide a hybrid meeting in 2022 and ask you to encourage your family and friends who are affected by bleeding disorders, or who just want to learn more, to register and attend virtually. Information on current and future treatment therapies and bleeding disorders services are made available during our exhibit hall experience. Registration is required for attendance. All meals, sessions, and childcare

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are included with hotel accommodation available for our traveling guests. The event is free of charge for BDASC members. Registration information to join the online virtual sessions will be available closer to the meeting date. *Registration information will be available in the Spring of 2022.* We hope you will plan to join us! Our Agenda schedule:

5:30 p.m. — Friday Night Chapter Annual Meeting

9:00 a.m. — Saturday Sessions and Breakouts

5:30 p.m. — Dinner Wrap-up, Hotel Terrace

"We wanted to personally thank you for your continued generosity to support research through the Judith Graham Pool (JGP) research fellowship program. We cannot emphasize strongly enough how important your support is to the continued success of our JGP fellows and to the advancement of the innovative science they are pioneering. Your support directly impacts people living with inheritable blood disorders by accelerating the cutting-edge science that is revolutionizing treatments

and health care. We are truly grateful for your partnership and commitment to advancing research and the careers of the promising researchers that you support.

Today's support is making tomorrow's medicines a reality right now. Supporting the JGP is so critical to ensuring that innovative science continues to advance, and that innovative scientists are able to launch their hematology careers. Your whole-hearted support for the mission and your enthusiastic engagement is equally critical to our collective success. I wanted to express my deepest gratitude for making me feel so incredibly welcomed into the organization and the community. I've truly found my home here!"

— Kevin Mills, NHF, CSO



BDASC at the Inspiring Shots Golf Tournament

The Inspiring Shots Golf Tournament was held Monday, December 13, 2021, at Rosen Shingle Creek in Orlando, FL, for the second straight year. Sponsored by the Wingmen Foundation, BDASC was represented by Hunter Day, Joey Krakowiak, and Matt Weber. Taking on the Utah Hemophilia Foundation, Hemophilia Foundation of Minnesota/Dakotas, and New England Hemophilia Foundation, the Carolina crew played some great golf. The team had a blast and did BDASC proud. Thank you to the Wingmen Foundation,



The Preserve at Verdae, Perry Parker, and CSL Behring for providing our Chapter members this fabulous

opportunity to learn about golf, enhance their skills, and enjoy the game!

Community Connections and Support Groups Outreach

November 29—Community Connections Virtual Program —Empowered: Tools for Self-Advocacy

BDASC and Pfizer held a Community Connections Virtual Program, “Empowered: Tools for Self-Advocacy,” with a focus on self-advocacy. This program also included a helpful module on health insurance and how

it is a critical component of self-advocacy. We enjoyed gathering online together. A huge appreciation to Pfizer for the partnership opportunity.



November 30—“On the Road” Healthcare Insurance Roadmap at Rioz

BDASC held an educational outreach dinner at Rioz Steakhouse in Columbia to celebrate the holiday season learning about the Healthcare Roadmap. The Healthcare Insurance Roadmap was a presentation that provided individuals with the information that they need to navigate healthcare coverage. The topics “Understanding Healthcare Coverage” and “Selecting a Health Insurance Plan” were covered as part of the session. Since insurance is not the most exciting subject, the Chapter



also held an Ugly Sweater Contest, which included a couple of electrifying participants! This evening was supported by Takeda, and we appreciated the opportunity to connect.



December 9—Singles and Couples Holiday Support Event

In Greenville, the BDASC held a fun night out for adults and couples. This small support group activity was designed to help members unwind from the hustle and bustle of the holiday season while creating a project at Board and Brush Creative Studio. The speaker at the event was Kim, a patient advocate affected by Von Willebrand Disease who talked on “Channeling your emotions into advocacy.”

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Educate through awareness.” The event was a fun and memorable time for all who attended. This event was supported by CSL Behring, and we appreciated the opportunity to gather, eat, learn, and become creative.



January 13—MEN'S Virtual Meeting

The MEN'S program had a special guest speaker for their first meeting of the year, Performance

Business Coach Justen Martin. A husband and father whose family was dealt a devastating, life-threatening diagnosis, explained how he worked from rolling burritos to making millions in real estate. In his roller coaster of life, he learned that you have to put in the work and make strategic sacrifices to get what you really want: personal happiness! His talk applied not just to business, but life. The MEN'S program planning for 2022 was also discussed.

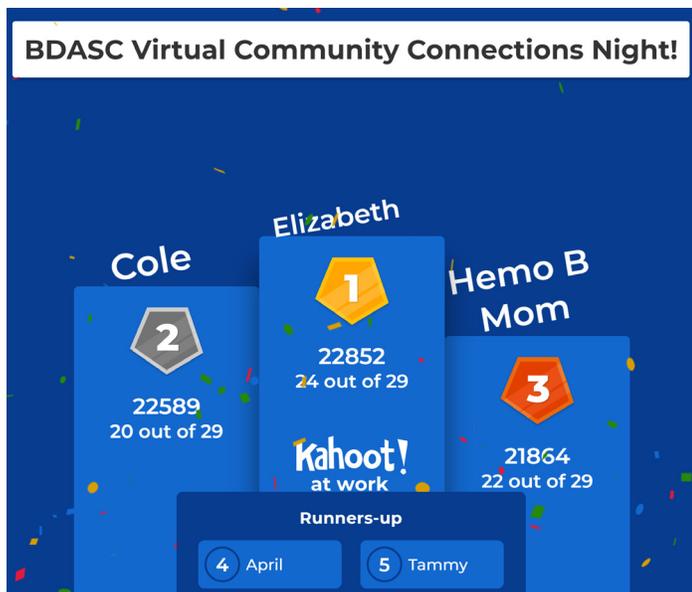
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January 17—Virtual Community Connections Kahoot!

The community gathered together for the first meeting of the year in January and played an informative game of virtual Kahoot and enjoyed eating pizza together. Information on the rollout of the new website, the outcome reports of the member feedback survey, along with details of upcoming programs and events, including service support, were all part of the Kahoot questions. It was fun to see everyone after the long holiday season break. We wish to congratulate Elizabeth, Cole, and Coree for their excellent play.



March 1-2—NHF Washington Days (Virtual)

NHF will be hosting a virtual Washington Days this year on March 1-2. Our members, Advocacy Coalition, and Ambassadors will be in attendance with the bleeding disorders community throughout the nation. Please visit the *Advocacy Action* section for more details.

March 8-9—BDASC's Advocacy Days (Columbia)

BDASC will be holding its 13th Annual Advocacy and State Legislative Days on March 8 and 9. See the *Advocacy Action* section for more details.

March 25-27—BDASC's Women's Bleeding Disorders Conference (Greenville)

BDASC will be holding its first-ever conference intended for women who are affected by

hemophilia, Von Willebrand Disease, or other rare bleeding and platelet disorders. It will be held on March 25 to 27 at the SpringHill Suites in Greenville. The deadline to register is March 2. See the feature article about the conference for more information.

April 15—World Hemophilia Day Celebration (Greenville)

Since World Hemophilia Day falls on Easter, we will be celebrating the day on Friday, April 15, in Greenville and again for our Men's Program in Columbia on the 16th! Come enjoy these days of celebration with us as we end our Red Tie Campaign and recognize World Hemophilia Day! Hear from our partners within the bleeding disorders community and show your support. Together, we will continue to build one strong community! This event is held in partnership with Sanofi Genzyme. Check our website and social media for more details about this event as they develop.

April 20-23—HFA Symposium (Houston)

HFA's in-person Symposium will be April 20-23, 2022. See the *National News* section for more information.

May 14—STEP for Bleeding Disorders (Saluda Shoals Park, Irmo)

Our STEP for Bleeding Disorders will be on May 14, 2022, at Saluda Shoals Park. See the featured article for more information.

June 10-11—BDASC Annual Meeting & HELLO Conference

The Annual Meeting and HELLO Conference will be held June 10-11, 2022, at the Embassy Suites by Hilton Greenville Golf Resort & Conference Center. See the featured article for more information.

Bleeding Disorders Awareness Campaign "Red Tie Campaign" Activities Proclamations

The Red Tie Campaign is upon us! As our Chapter's Annual Giving Drive, the Red Tie Campaign is held every March as part of the National Bleeding Disorders Awareness Month.

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Funds raised during the campaign help Bleeding Disorders Association of South Carolina to support research, community advocacy, education, and access to quality treatment. All donations to our Red Tie Campaign stay within South Carolina to serve our community. BDASC's Red Tie Campaign runs from Rare Disease Day, February 28, through World Hemophilia Day on April 17. As part of the event, BDASC will be holding its 13th Annual Advocacy and State Legislative Days on March 8-9 and our World Hemophilia Day Celebration on April 15-16. We will once again be gathering Proclamations from towns, cities, and counties and encouraging everyone to **"Go Red" for St. Patrick's Day** on March 17.

How do you secure a proclamation? You can visit the proclamation page on the Red Tie Campaign Website for instructions and all the needed documents. Find your local Town/City/County's contact person, often a clerk or administrative assistant. If you need help with this, please call the Chapter. If approved, let us know so we can send you any additional materials and notify other members that live in your area. And don't forget to take plenty of pictures so we can add them to our newsletters, e-blasts, and website! One of our biggest efforts during the campaign will be attending NHF's virtual Washington Days, March 1-2, and hosting our own in-person State

Days March 8-9. This year our fundraising goal is \$5,000, and every single donation goes to the betterment of the Chapter's members. A \$50 donation helps provide financial assistance so members can attend educational and supportive events; \$250 assists members so they can attend clinic days at the Hemophilia Treatment Center; and \$1,000 helps provide our bus services so our teens can attend our famous Teen Retreat. In this Campaign, all the donations support the programs and services you know and love that the Chapter provides. For more information, visit our donation site at: <https://secure.qgiv.com/event/redtie2022/>

Put a Little STEP in Your Spring This Year!

After a two-year transition, autumn's Turkey Trot is now spring's STEP for Bleeding Disorders. If you were with us for the 2021 STEP, you know one of the big reasons we're moving the event is that it can get cold in November, even here in South Carolina! We want you, your family, and all your friends to register and join us on May 14, 2022, for our 9th Annual STEP for Bleeding Disorders 5K Walk / Run Fundraiser and Awareness Campaign. We will once again be holding our walk in Columbia at Saluda Shoals Park.

What is STEP for Bleeding Disorders and what is its meaning? The word STEP encompasses everything the Bleeding Disorders Association

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Continued from page 13 of South Carolina holds to its core. **S** is for Sharing Stories, **T** is for Transforming Tomorrows, **E** is for Empowering People, and **P** is for Promoting Research. We continue to be determined to raise awareness and support for our community in South Carolina, along with research for better treatments (and someday a cure) and support for world initiatives for our global bleeding disorders family. How can you STEP? You can join yourself, form a team, join an existing team, or simply make a donation to the cause. Visit <https://secure.ggiv.com/event/sfbd22/> for registration information and all the steps to register.



Come meet our community and help us raise funds too! Any amount you can raise will help make a difference in the lives of our members. So, register today and join us on May 14 at 9:00 a.m. as we join the community and STEP into action and Walk / Run together! All state and CDC COVID-19 guidelines will be respected for a safe and fun event for all.

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Voices of The Community

Meet Wendy Legrand, Advocacy Coalition member

My name is Wendy Legrand. I am married and have a 12-year-old son with severe Hemophilia A. I have been on the Board of Directors for four and a half years, and I'm also on the Advocacy Coalition where I started out being the secretary for several years. I have had the chance to go to Washington, D.C., and have gone to the state house for legislative days several times. This year as we all get ready next month to go to the Capitol, I sit back and think about what I need to tell my representative and senator. We all have different struggles with living and caring for ourselves or someone we love with a bleeding disorder but this year my struggles are different

than what they have been. In the past, I've gone to the Capitol and just shared my story of what it's like raising a child with a bleeding disorder. Now I worry about making sure we have good health insurance. I worry about copays, deductibles and what out-of-pockets are going to be. I worry that my small company will drop us because of the cost of his medicine. I worry that he could lose his insurance. I worry that he won't be able to get the medicine that he needs. As a parent I worry about this all the time. We need everyone to come to Capitol Hill and voice your concerns. Tell your story. Be an advocate for yourself and your family! —*Wendy Legrand*

Von Willebrand Community

Clinical Study and New Website to Focus on von Willebrand Disease and Pregnancy

The onset of childbirth and the postpartum period are times when women with von Willebrand disease (VWD) are at an increased risk for excessive bleeding, exposing them to further, and in some instances, serious complications. While there exist therapies with VWD-specific indications, it is not uncommon for these patients to still experience excessive bleeding while receiving treatment. These scenarios are challenging as there is sparse clinical data and a subsequent lack of clear guidance on the optimal management of bleeding in these particular settings. The von Willebrand factor in pregnancy (VIP) study was therefore developed to enhance understanding of how best to manage bleeding during delivery and the postpartum period in women with VWD. Investigators for this prospective, multicenter trial will focus on maintaining von Willebrand factor (VWF) levels

at a specific target level using VWF replacement therapy and assessing the impact on bleeding rates during and after childbirth.

The VIP study is being stewarded by a trio of experienced principal investigators including Drs. Jill Johnsen (Bloodworks and University of Washington), Barbara Konkle (University of Washington), and Dr. Peter Kouides (Mary M. Gooley Hemophilia Center and University of Rochester). The VIP Study is currently recruiting pregnant women in the U.S. above 18 years of age with VWD of any type.

An exciting component of the VIP is a new companion website, created to keep patients and healthcare professionals informed about the study. It will provide information on VIP's design, patient eligibility, and locations of participating centers. The site was officially launched on December 10 during an educational webinar that preceded the American Society of Hematology Annual Meeting.

Source: PR Newswire/The VIP Study, November 28, 2021

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 Treatment Center Network (USHTCN) and the Centers for Disease Control and Prevention conducted 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 were published 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 months earlier than in those without such a history. In all, family history of a bleeding disorder prompted diagnostic testing for 68%

Continued on page 20

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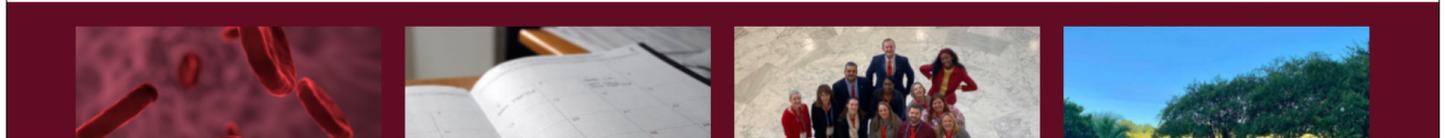
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Lower Patient Copay Act Advances

More than 140 groups representing patients with serious and chronic conditions this week sent a letter to House Representatives Donald McEachin (D-VA) and Rodney Davis (R-IL) voicing support of their recent introduction of H.R. 5801, the Help Ensure Lower Patient Copays Act (HELP Copays Act). The legislation, which included seven additional co-sponsors from both sides of the aisle, is a two-part solution that helps ensure patients can cover their out-of-pocket costs and access needed medications.

[read article >](#)



The New BDA-SC.org Website Is Live!

After a year of development, BDASC launched our new website with much fanfare on Friday, December 31. Designed by Richard Wright and under the supervision of Sue, the new domain at BDA-SC.org is more integrated, better optimized for mobile use, and is smoother in design than the previous site. With many of the same sections and categories as the old one, the new site allows you to now register for events on their information page. It will also have an easier-to-access news blog to stay up to date on late-breaking Bleeding Disorder news. We invite you to explore all it has to offer and hope the website proves to be a resource for all things Bleeding Disorders!

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, and 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

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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 the associated 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 resented 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 highest quality 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 -dovepress.com/integrated-hemophilia-patient-care-via-a-national-network-of-care-cent-peer-reviewed-fulltext-article-JBM

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.

BDASC Featured in Greenville, Columbia, and Charleston Business Magazine



If you picked up this December's issue of the Greenville, Columbia, and Charleston Business Magazine and browsed through the Special Giving Guide, you would have seen BDASC's very own half-page ad on page 84! Explaining to the public the importance of our organization

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and what it's like to live with a chronic bleeding disorder, this ad put BDASC in front of thousands of readers, across the state and beyond. With so many new people learning about us, we hope to reach many new families who may need our services and don't realize we are here to serve the bleeding disorders community in South Carolina. This year alone, we have added 56 new families into our organization with the help of referrals from our HTC's and our website. You can still read the entire Magazine online at tinyurl.com/GBMBDASC.

BDASC Mourns the Loss of Longtime NHF CEO, Val Bias

BDASC is deeply saddened by the announcement that former NHF CEO and community member, Val Bias, passed away suddenly on Thursday, December 30, 2021.

BDASC will forever honor Val's legacy and all that he accomplished for the inheritable blood disorders community in the United States and internationally. Most remarkably, Val was a uniquely skilled community builder, creating coalitions and cultivating champions for our community. Prior to his tenure as CEO of NHF, Val served as Chairman of The Board of Directors of NHF from January 1992-January 1994. He also served in multiple capacities on the global stage working closely with the World Federation of Hemophilia and advancing the International Twinning Program. Val transformed the NHF chapter network, unifying and strengthening our voice and impact from the statehouse to the halls of Congress. His many notable contributions included establishing the first NHF Washington Days and championing the National AIDS Memorial's Hemophilia Circle. As CEO he led NHF into a new era of growth and service, he stabilized NHF funding and built programs to serve those who had been left behind. His lifelong commitment to families and youth was no more evident than through his commitment as a summer camp counselor,



programmer, in his service as Camp founder and Staff Director of Camp Hemotion: Camp for Children with Bleeding Disorders. He began his career with NHF in Washington championing the passage and funding of the Ricky Ray Act and working to transform the regulatory framework to ensure the tragedy of HIV/AIDS would not repeat. His impact of his forceful advocacy will long be felt, from the halls of power at the U.S. Capitol, to the arts and crafts room at local summer camps. "The reason NHF and the inheritable blood disorders community has come so far is because of what Val was able to accomplish," said NHF President and CEO Len Valentino. "I had the honor to walk alongside Val both professionally and personally and can say with certainty that what he has done throughout his life inspired change. His legacy will live on forever, and he will be greatly missed."

Mother of Ryan White, Jeanne-White Ginder, said in tribute "I am most grateful to Val for including me in the hemophilia community and widening the circle to make sure everyone's voice was heard. He made sure our losses were not forgotten, especially through his efforts to include the hemophilia community in the National AIDS Memorial Grove. Val knew the hemophilia community because he lived hemophilia alongside all of us. In spite of his own health battles and personal losses, he carried on with courage and strength that inspired us all, making him not only an accomplished leader but a role model that will never be forgotten."

BDASC, NHF, and the community are heartbroken over this sudden loss. Val served as NHF's CEO for over a decade and carved the path for the organization's continued growth and sustainability. NHF extends sincere condolences to Val's beloved son, Langston, his loving wife of twenty years, Robin, and his entire family.





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 Carolyn Tyler
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 Ric and Sue Martin

In Memory of Rodney Hawley

Loretta Engelhardt

In Honor of Cristal and Hunter Day

Carolyn and Roy Ross

In Honor and Memory of Roy Clifford Sperry

David and Bonnie Ashmore
 Gregory and Cherron Saad
 Jennifer Sperry: Developmental Services and Staff
 Karen Richardson

Kitty Wright
 Lauri Ashmore
 Linda and Richard Ashmore
 Mark Ashmore
 Sandra Hunnicutt and Randall Griffin
 Jean and Donna Whitlock

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 National Hemophilia Foundation
 Octapharma
 Paragon Specialty Pharmacy
 Pfizer
 PhRMA Foundation
 Takeda Pharmaceuticals

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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 HTC's 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 HTC's 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

Advocacy Action

State Advocacy Days: A Time to Educate

BDASC will be holding its 13th Annual Advocacy and State Legislative Days March 8–9 as part of National Bleeding Disorder Awareness Month. This event will be held live with virtual meetings available for those unable to attend in person. We will be requesting proclamations throughout the state recognizing this special awareness month and attending event ceremonies as available. BDASC, along with our national partners, will be hosting an advocacy training session leading up to our state days. This important advocacy event fosters educational awareness with state legislators on the effects living with a bleeding disorder can have on individuals and families. You will have the opportunity to have important discussions and to ask for support on key areas of concern for access to affordable treatment. Since its inception, our State Capitol Advocacy Days have provided for over 500 individual meetings with members of the South Carolina House of Representatives and Senate from districts throughout the state. These meetings are of key importance in helping our elected officials understand our community's unique needs, how access to medications and treatment are critical, and for support of our state programs that provide a safety net to those in need. Health care access is continuously challenging, including the barriers to high-cost medications needed by individuals with bleeding disorders. Only you can help remove these barriers with your advocacy participation! This event is for the whole family with teen programming included. Thank you for raising your voice!

Advocacy training is provided in partnership with our national organizations and the South Carolina Hemophilia and Bleeding Disorders Advocacy Coalition and Ambassadors, The National Hemophilia Foundation, Hemophilia Federation of America, the National Organization for Rare Diseases (NORD), our stakeholders, and

other local and national advocacy partners. Registration is required for meetings and hotel stays. All food, meeting space, and educational materials are included for members

of BDASC. Registration is open so please do so today on our website. Thank you! <https://bda-sc.org/state-capitol-days/>



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National News

Hemophilia Federation of America Symposium 2022

The Hemophilia Federation of America is heading to San Antonio for what is shaping up to be their biggest and best Symposium yet. Mark your calendars April 20–23, 2022, and plan to join them deep in the heart of Texas for the first in-person Symposium since 2019. Registration is open at hfasymposium.org.

NHF's Bleeding Disorders Conference 2022

Texas is the place to be this year as NHF will be hosting the Bleeding Disorders Conference 2022 in Houston. Running from August 25 to 27, you can find more information about the event at hemophilia.org. If you are a young adult between the ages of 18–35 and you are interested in attending, don't forget to apply for the

Joe Caronna Memorial NHF Bleeding Disorders Conference Scholarship. BDASC will grant one young adult who has a diagnosis of hemophilia, von Willebrand Disease, or other bleeding disorder, and is a current Chapter member, a scholarship to attend. The scholarship provides meeting hotel room costs for three nights—Thursday, Friday, Saturday—during the meeting, a travel grant up to \$350, and paid registration, for a total not exceeding \$1,500. Remember, to qualify you must be an active registered member of BDASC, have a confirmed diagnosed bleeding disorder, be able to travel alone or with a spouse, family member, or other at their own expense. Please fill out the full application form and submit the required essay. For more information and to access the application, please visit <https://bda-sc.org/national-education-and-conferences-travel-scholarships>

Virtual NHF Washington Days

NHF will be hosting a virtual Washington Days this year on March 1–2. It is the best opportunity for people affected by bleeding disorders to advocate for issues that are important to the bleeding disorders community. The Bleeding Disorders Association of South Carolina has participated each year providing a voice for South Carolina's bleeding disorders community and a strong unified commitment to the national community. Last year a record 500 attendees from 47 states and Puerto Rico gathered virtually for the community advocacy event. Volunteer advocates met with legislators and staff to discuss federal funding for bleeding disorders programs and access to skilled nursing facilities (SNFs).

For this year's event to be as successful, we need to make sure we have at least one member from each of South Carolina's seven congressional districts. To ensure you are properly prepared, each participant of Washington Days is provided a briefing and training session held the day before the Capitol visit. You will be given talking points

and briefed on the key issues that we will be discussing. One of the big bills we are fighting for is HR 5801, the "Help Ensure Lower Patient Copays Act" or the "HELP Copays Act." This act would restrict copay accumulator adjustment programs by ensuring that all insurers "apply additional payments, discounts, and other financial assistance towards the cost-sharing requirements of health insurance plans, and for other purposes."

Washington Days is a great experience for everyone and, if you are attending, contact the Chapter so we know who all is representing our state and Bleeding Disorders Association of South Carolina.



Research & Development

NHF's Community Voices in Research

BDASC is working in partnership with the National Hemophilia Foundation to advance patient care, treatment, and research. We believe strongly in making advancements that will help our community thrive in the years ahead. We will share these opportunities with you when presented to us allowing you to make an informed choice to have a hand in the Community Voices in Research (CVR).

Throughout 2022, we will continue to share with our community members information about Community Voices in Research (CVR). Based on current data, we have learned that:

- 58% of women with bleeding disorders have a history of bleeding before or after childbirth;
- 19% of men experience pain most days or every day. That number jumps to 39% when we look at women;
- 35% of participants reported that their bleeding disorder had caused them to have difficulty maintaining employment.

We will have the opportunity to get access to reliable aggregated data for our chapter's future programming and our community

members will benefit from informing the current priorities in research and what it means to live with an inherited bleeding disorder from their perspective. We invite you to learn more and participate if you are interested.

VAP Invite: We need your input!

As a valued member of the bleeding disorders community, you are invited to participate in one of the upcoming *Virtual Advisory Panels (VAPs)*:

Understanding Challenges of Patient Engagement in Therapy Management. A VAP is an opportunity to provide your thoughts directly to NHF and to sponsors working on specific projects related to the bleeding disorders community. The

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SHARE YOUR VOICE!

Only 6% of CVR participants are Black/African American. Among them, 56% identify as female and 44% as male. Your lived experiences are important to shape the future of research.

Community Voices in Research
NATIONAL HEMOPHILIA FOUNDATION

WWW.HEMOPHILIA.ORG/CVR

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purpose of *this* VAP is to take a closer look at therapy management and identify the challenges and barriers patients face in order to help improve and design future programs provided to the bleeding disorders community. We want to hear from YOU!

Please take 3–5 minutes to help us determine your eligibility for this upcoming VAP. If you meet the criteria, *Felix Olaya, MPH Research Recruitment & Engagement Specialist* from NHF's Research department will reach out to you with further information, and those selected will be compensated for their time.

To complete your survey please use this link:

https://survey.az1.qualtrics.com/jfe/form/SV_9mhoxHpXBS0irlA

uniQure and CSL Behring Announce Primary Endpoint Achieved in HOPE-B Pivotal Trial of Etranacogene Dezaparvovec Gene Therapy in Patients with Hemophilia B

Lexington, MA and Amsterdam, the Netherlands, King of Prussia, PA, December 9, 2021 — CSL

Behring, a global biotherapeutics leader, and uniQure N.V., a leading gene therapy company advancing transformative therapies for patients with severe medical needs, today announced that etranacogene dezaparvovec, an investigational adeno-associated virus five (AAV5)-based gene therapy for the treatment of patients with severe to moderately severe hemophilia B, achieved the pre-specified primary endpoint of non-inferiority in annualized bleeding rate (ABR) 18-months following administration compared to baseline Factor IX (FIX) prophylactic therapy in the pivotal Phase III HOPE-B gene therapy trial. The study also successfully achieved a secondary endpoint demonstrating statistical superiority in reduction of ABR compared to baseline FIX prophylactic therapy.

The primary endpoint in the pivotal study was 52-week ABR after achievement of stable FIX expression compared with the six-month lead-in period, considering all bleeds regardless of

investigator adjudication as true bleeds. For this endpoint, ABR was measured from month seven to month 18 after infusion, ensuring the observation period represented likely steady-state FIX transgene expression. Secondary endpoints included assessment of FIX activity and statistical superiority of ABR after dosing. “We are very pleased with these top-line results from what is the largest and first pivotal trial of a gene therapy for patients with hemophilia B,” stated Ricardo Dolmetsch, Ph.D., president of research and development at uniQure.

“The HOPE-B data not only achieved the pre-specified primary endpoint of non-inferiority in annualized bleeding rate following 12 months or more of stable FIX expression, but also the secondary endpoint of superiority in reduction of annualized bleeding, while continuing to demonstrate durability and stability in FIX levels and other benefits to this point in the study.”

“On behalf of uniQure, we extend our heartfelt gratitude to all the HOPE-B clinical trial patients and their families, as well as the trial investigators,” he continued. “We now look forward to collaborating with CSL Behring on completing the regulatory submissions that we hope will advance etranacogene dezaparvovec one step closer to reaching hemophilia B patients around the world.”

uniQure led the multi-year clinical development of etranacogene dezaparvovec prior to entering into a Commercialization and License Agreement with CSL Behring in June 2020 for exclusive global rights to etranacogene dezaparvovec. Earlier this month, uniQure successfully completed manufacturing operations supporting process validation of etranacogene dezaparvovec.

Etranacogene dezaparvovec has been granted Breakthrough Therapy Designation by the United States Food and Drug Administration and access to Priority Medicine (PRIME) regulatory initiative by the European Medicines Agency. CSL Behring plans to submit regulatory applications for marketing approval of etranacogene dezaparvovec in the United States and European Union in the first half of 2022.

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Top-line Data Results

A total of 54 patients received a single dose of etranacogene dezaparvovec in the pivotal trial, with 53 patients completing at least 18 months of follow-up ABR for all bleeds after stable FIX expression, assessed at 18 months, was 1.51 compared with the ABR of 4.19 for the lead-in period of at least six months, achieving the primary non-inferiority endpoint and a secondary superiority endpoint ($p=0.0002$) in the HOPE-B trial. ABR for investigator-adjudicated FIX-treated bleeds was 0.83 compared with lead-in ABR of 3.65 ($p<0.0001$). Data from the HOPE-B pivotal trial showed that patients continued to demonstrate durable, sustained increases in FIX activity at 18 months post-infusion with a mean FIX activity of 36.9 percent of normal as measured by a one-stage APTT-based clotting assay, compared to mean FIX activity of 39.0 percent of normal at six months post-infusion.

Etranacogene dezaparvovec was generally well-tolerated with over 80% of adverse events considered mild. One death resulting from urosepsis and cardiogenic shock in a

77-year-old patient at 65 weeks following dosing was considered unrelated to treatment by investigators and the company sponsor. A serious adverse event of hepatocellular carcinoma (HCC) was identified in one patient. Independent molecular characterization and vector integration analysis of the HCC and adjacent tissue supported the conclusion by the investigator and company sponsor that the HCC was unrelated to treatment with etranacogene dezaparvovec. No inhibitors to FIX were reported.

“These encouraging results illustrate the potential that gene therapy has to be a long-term treatment option for patients living with hemophilia B and we look forward to sharing more detailed data with the medical community in the near future,” stated Brahm Goldstein, MD, MCR, Vice President, Research and Development, Hematology at CSL Behring. “This milestone advances our efforts towards expected regulatory submissions in first half of 2022.”

A more detailed version of this article can be found in the News Blog of our website, [BDA-SC.org](https://www.bda-sc.org). New articles can be found there regularly.

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News

Download the 2022 PDF Calendar of Events: [CLICK HERE](#)

PLEASE READ AND VIEW OUR COVID – 19 MITIGATION POLICY AND WAIVER RELEASE TO ATTEND LIVE EVENTS: [CLICK HERE](#)

Clinical Study And New Website To Focus On Von Willebrand Disease And Pregnancy
December 30, 2021

The onset of childbirth and the postpartum period are times when women with von Willebrand disease (VWD) are at an increased risk for excessive bleeding, exposing them to further, and in some instances, serious complications. While there exist therapies with VWD-specific indications, it is not uncommon for these patients to still experience excessive bleeding while receiving treatment. These scenarios are challenging as there is sparse clinical data and a subsequent lack of

Study In Hemophilia B Achieved Primary Endpoint Of Non-Inferiority
December 20, 2021

Largest gene therapy study in hemophilia B achieved primary endpoint of non-inferiority in annualized bleeding rate after stable Factor IX (FIX) expression, assessed at 18 months following a single dose of etranacogene dezaparvovec. Etranacogene dezaparvovec also achieved secondary endpoint demonstrating statistical superiority in reduction of annualized bleeding rate compared to baseline FIX prophylactic therapy. Stable and durable FIX levels with mean FIX activity of 36.9 percent of normal in full study

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Resources & Contact Information

Medical Facilities

The Hemophilia Treatment Center of South Carolina

Children's Cancer & Blood Disorders Clinic at Prisma Health Children's Hospital—Midlands

7 Richland Medical Park Rd., Suite 7215
Columbia, SC 29203-6872
Phone: 803-434-3533

Stephanie Phillips Ambrose, MD, HTC Director
Caitlin Sullivan, LMSW, HTC Social Worker

For all new and previously scheduled appointments, factor refills, school needs, general questions or general concerns: Contact Robin Jones, MSN, MHA, RN, CPN, CPHON Children's Cancer and Blood Disorders Nurse Navigator/ SC Hemophilia Treatment Center Nurse Coordinator at 803-434-1028 or email @ schemophilia@prismahealth.org. You may leave a message and your call will be returned as soon as possible. Robin may be away from her desk or with a patient in the clinic.

Hemophilia Treatment Center — Satellite Office (Greenville)

Prisma Health – Upstate
BI-LO Charities Children's Cancer Center
900 W. Faris Road, 2nd Floor
Greenville, SC 29605
Phone: (864) 455 - 8898
Fax: (864) 455 - 5164

Leslie Gilbert, MD MSCI, Director
Diana Moreno, RN, HTC Nurse
Pamela Broughton, LISW, HTC Social Worker

Medical University of South Carolina Shawn Jenkins Children's Hospital

10 McClennan Banks Dr.
Charleston, South Carolina 29425

Shayla Bergmann, MD, Assistant Professor
Director, Pediatric Hemophilia Clinic

Phone: 843-792-2957
Mobile: 843-812-5682
Clinic: 843-985-1667
Clinic scheduling: 843-876-0444
Fax: 843-985-4255

Local and National Orgs

Bleeding Disorders Association of South Carolina

Green Gate Office Park
25 Woods Lake Road, Suite #300
Greenville, SC 29607
Phone: 1-864-350-9941
Fax: 864-244-8287
Email: info@bda-sc.org
Web site: www.bda-sc.org

National Hemophilia Foundation

7 Penn Plaza, Suite 1204
New York, NY 10001
Phone: 212-328-3700
Fax: 212-328-3777
Phone: 800- 42-HANDI (4-2634)
Fax: 212- 328-3799
Email: handi@hemophilia.org
Website: <http://www.hemophilia.org/>

Hemophilia Federation of America

999 North Capitol Street, NE, Suite 201
Washington, DC 20002
Phone: 800-230-9797
Email: info@hemophiliated.org
Website: www.hemophiliated.org

World Federation of Hemophilia

1425, boul. René-Lévesque O.
Bureau 1010
Montréal, Québec
H3G 1T7 Canada
Phone: +1 (514) 875-7944
Fax: +1 (514) 875-8916
Email: wfh@wfh.org
Website: www.wfh.org

Resource Information

SC Dept. of Health & Environmental Control (DHEC)

Children with Special Health Care Needs
Hemophilia Program
2100 Bull Street
Columbia, SC 29201
Phone: 803-898-0784
Website: <https://www.scdhec.gov/health/child-teen-health/services-children-special-health-care-needs/hemophilia-assistance-plan>

American Pain Foundation (APF)

Phone: 1-888-615-PAIN (7246)
Hemophilia Chronic Pain Support Group
Website: <http://painaid.painfoundation.org>

American Society of Pediatric Hematology/Oncology

Phone: 847-275-4716
Website: www.aspho.org

The Coalition for Hemophilia B, Inc.

Phone: 212-520-8272
E-Mail: hemob@ix.netcom.com
Website: www.hemob.org

Bleeding Disorders Legal Hotline

Phone: 800-520-6154

Centers for Disease Control & Prevention

Phone: 1-800-311-3435
Website: www.cdc.gov

Committee of Ten Thousand (COTT)

Phone: 800-488-2688
Website: www.cott1.org

LA Kelley Communications, Inc.

Phone: 978-352-7657
Website: www.kelleycom.com

Patient Access Network Foundation

805 15th Street, NW, Suite 500
Washington, DC 20005
Phone: 202-347-9272
Website: <https://panfoundation.org/index.php/en/>

Helps underinsured people with life-threatening, chronic and rare diseases get the medications and treatments they need by assisting with their out-of-pocket costs and advocating for improved access and affordability.

Patient Notification System

The Patient Notification System is a free, confidential, 24-hour communication system providing information on plasma-derived and recombinant analog therapy withdrawals and recalls.

Phone: 888-UPDATE U (873-2838)
Website: www.patientnotificationsystem.org

Patient Services Incorporated (PSI)

Assists persons with chronic medical illnesses in accessing health insurance and pharmacy co-payment assistance.
Phone: 800-366-7741
Website: <https://www.patientservicesinc.org/>

Medic Alert Foundation

2323 Colorado Avenue
Turlock, CA 95382
Phone: 800-432-5378
Website: www.medicalert.org

South Carolina Healthcare Market Place

Call 800-318-2596
Website: www.HealthCare.gov

Upcoming BDASC Community Events

Mark Your Calendar!

Looking Ahead: Save the Dates! 2022 Major Event Dates

March 8-9—State Advocacy Days, Columbia State House, SC

March 25-27—BDASC's Woman's Bleeding Disorders Conference, Greenville, SC

May 14—BDASC STEP for Bleeding Disorders, Columbia, SC

June 10-11—Annual Hello Conference and Annual Meeting, Greenville, SC

August 11-14—Teen Retreat, Camp Canaan, Rock Hill, SC

October 7—Par for the Clot Charity Golf Tournament, Greenville, SC

December 11—Winter Statewide Meeting, Columbia, SC

Additional ways to help BDASC



Go to www.bda-sc.org and click "Donate Now" to learn more about how you can support BDASC!



Register with Amazon Smiles. BDASC receives 5% of the sale! Go to <https://smile.amazon.com/>



Donate through United Way! Go to <http://www.unitedway.org/>



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