The News Infusion

The Official Newsletter of Bleeding Disorders Association of South Carolina

Volume 15, Issue 1

Winter 2025

bleeding disorders awareness campaign

SUPPORTING THOSE LIVING WITH BLEEDING DISORDERS
FEBRUARY 28TH - APRIL 17TH

VON WILLEBRAND DISEASE
awareness day
engage. empower. educate
MARCH 1st

ULTRA RARE BLEEDING DISORDERS
awareness day
rare factor & platelet disorders
MARCH 15th

WOMEN WHO BLEED
awareness day
engage. empower. educate
MARCH 30th

World
Hemophilia Day
April 17
Recognizing all bleeding disorders

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About BDASC & The News Infusion

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BDASC 2025 Staff & Board of Directors

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- A Chapter Member of the Hemophilia Federation of America
- A Registered Greenville County Charitable Organization
- A Member of the Greenville Chamber of Commerce
- A Member of the South Carolina Associations of Non Profit Organizations - Together SC
- A Member of the SC Palmetto Health Collective

BDASC Mission: Our mission is 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: Our vision 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.

Looking Ahead: Save the Dates!
Winter/Spring 2025 Calendar of Events

February 25-26th: *State Legislative Days, Columbia, SC*
March 1st – 31st: *Bleeding Disorders Awareness Month*
March 1st: *VWD Awareness Day Virtual Event*
March 15th: *Rare & Platelet Disorder Awareness Day Virtual Event*
March 17th: *Go Red for St. Patrick's Day Event*
March 30th: *Women Who Bleed Awareness Day Virtual Event*
April 4 - 6th: *Adult Connections Retreat, Greenville, SC*
April 17th: *World Hemophilia Day Event, Greenville SC*
April 19th: *Young & New Families Event, Greenville, SC*
April 24th: *Couples Night, Charleston, SC*
May 10th: *STEP for Bleeding Disorders Walk/Run, Columbia, SC*
May 15th: *Community Engagement Dinner Connections*
May 17th: *T.E.A.M Teen Transition Event*
June 6-7th: *Annual Meeting & HELLO Conference, Greenville, SC*
June 17th: *Community Engagement Dinner Connections*

About this Publication

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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 it's 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 healthcare 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 centers and the National Bleeding Disorders 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, Ste. 300, Greenville, SC 29607, Attention: Editor-in-Chief, The News Infusion. We look forward to hearing from you soon!

2025 Bleeding Disorders Awareness Campaign

February 28th, 2025 - April 17th, 2025

The Bleeding Disorders Association of South Carolina's Annual Giving drive is held every year during the month of March as part of the Bleeding Disorders Awareness Month. Funds raised during the campaign help us support research, global initiatives, provide community advocacy and education, and help us secure access to quality treatment care. All donations to our Campaign stay within South Carolina to serve our community. BDASC's Bleeding Disorders Awareness Campaign runs from Rare Disease Day, February 28th through World Hemophilia Day on April 17th. For more information and to help us reach our $2,500 goal, please visit our campaign website from www.bda-sc. org or https://tinyurl.com/2025BDAM.

During our Bleeding Disorders Awareness Campaign, we are highlighting different groups within the Bleeding Disorders Community, each receiving a day of recognition. Last year, we introduced two new days, March 1st as von Willebrand Disease Awareness Day and March 15th as Ultra Rare Bleeding Disorders Awareness Day. This year, we are excited to add Women Who Bleed Awareness Day in support of the women within our community. Women in the bleeding disorders community are often underserved, and their symptoms are many times dismissed. We look forward to taking a day to recognize these women and to help bring awareness! Each day will feature social media content highlighting interesting facts about the bleeding disorder that is being celebrated. We will also be concluding each awareness day with a celebratory virtual gathering.

thank you!

We received 62 orders and raised $730.40!! Thank you for your support!

World Hemophilia Day
April 17
Recognizing all bleeding disorders

This Year, the World Federation of Hemophilia will be recognizing all those living with a bleeding disorder on April 17th, World Hemophilia Day. This year's theme represents the inclusive mindset of WFH and their mission to support all those living with bleeding disorders throughout the world. On April 17, 2025, the global bleeding disorders community will come together to celebrate World Hemophilia Day. This year's theme is "Access for all: Women and girls bleed too". Today, women and girls with bleeding disorders (WGBDs) are still underdiagnosed and underserved. The global bleeding disorders community has the power—and the responsibility—to change this. Through recognition, diagnosis, treatment, and care, the quality of life of women and girls will improve, and the bleeding disorders community will become stronger.

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WOMEN WHO BLEED
awareness day
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MARCH 30th

Go Red on St. Patrick's Day

Braza Do Sul Brazilian Steakhouse
410 Columbiana Dr.
Columbia, SC

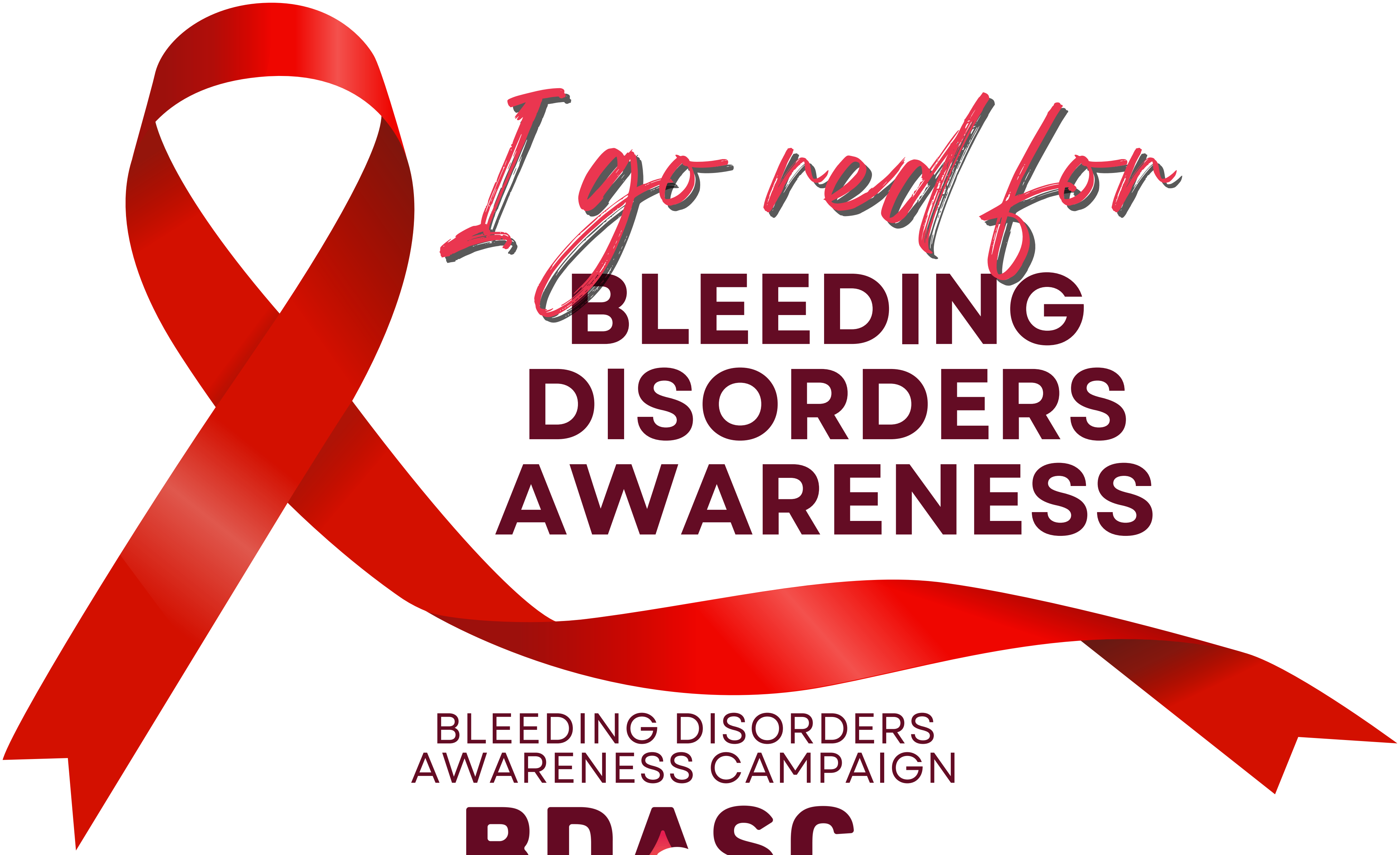
sponsored by: sanofi

Learn more and check out our upcoming Awareness Events by scanning the QR Code.

bleeding disorders awareness

SUPPORTING THOSE LIVING WITH BLEEDING DISORDERS

BDASC



I go red for

BLEEDING DISORDERS AWARENESS

BLEEDING DISORDERS
AWARENESS CAMPAIGN

BDASC

Executive Director's Welcome - A Heartfelt Message from Sue, Executive Director

By Sue Martin

As we welcome 2025, it's hard to believe that in less than a week, we'll be stepping into March—one of my most cherished months of the year. March is National Bleeding Disorders Awareness Month, and it brings with it an opportunity to celebrate the incredible strength of our community and to raise awareness about the challenges those living with bleeding disorders may face. It's a month that holds deep meaning for all of us, and I am so excited to see what we can achieve together this year. I look forward to connecting with familiar faces and meeting new ones as we unite to support each other and honor the journey we share.

As we wrapped up 2024, we were busy laying the groundwork for an exciting year ahead. We've carefully crafted our events and initiatives based on the feedback from you, our members—what you've expressed in surveys, at outreach meetings, and in the conversations we've had. Everything we have planned is designed with your needs in mind. I encourage you to take full advantage of the opportunities ahead—to connect with others, to learn, to grow, and to find the support and resources that will empower you. This is a time to strengthen the bonds within our community and to prepare ourselves, knowing that when the unexpected happens, we are never alone. Knowledge truly is power, and being part of this community means we can face whatever comes our way with a united front.

One thing I can never say enough is thank you. Thank you to every single person who has contributed, whether through a donation, a fundraiser, or simply showing up to our events. Every effort, large or small, has made a difference, and we are so deeply grateful. As we continue to grow, it's more important than ever that we come together to raise the funds necessary to keep our chapter strong for years to come. Your support ensures that we can continue to provide vital resources and advocacy to those who need it most.

On a personal note, I don't know where my family would be today without this community. Being connected to others who understood what we were going through made all the difference. It gave us the strength to face challenges, the comfort of knowing we weren't alone, and the assurance that there was always someone to turn to. Watching my children grow up with the knowledge that there were other kids like them—who shared the same experiences, struggles, and victories—was such a blessing. It's something I know many of you can relate to, and I hope you feel the same sense of belonging that has meant so much to us.

I'm also thrilled to share some exciting news: we have a new full-time member joining our team! Many of you already know Samantha Javorka, and I am beyond excited to announce that she has accepted the role of Engagement and Development Manager at BDASC. Samantha has been an invaluable part of our team for the past two years, and with our continued growth, it became clear that her position needed to expand. She will now focus on securing additional funding, organizing smaller-scale fundraisers, engaging our community digitally and locally in South Carolina, and working beside me supporting our ever-growing community. Please join me in welcoming Samantha to her new role! You'll be hearing from both of us as we move forward this year, and I can't wait to see what we accomplish together.

With our small but dedicated team, we always need the support of volunteers—whether it's at events or behind the scenes. If you have time to give, even if it's just a small commitment, I would be so grateful. As Samantha works virtually from the Summerville area, I'm still in need of boots on the ground in Greenville to help make our events run smoothly. Your time and energy are invaluable.

Finally, I want to express how incredibly honored I am to serve as your Executive Director. Working alongside so many passionate individuals and being part of a community that supports and uplifts one another, is truly a privilege. We still have a long way to go in our mission of a world with no bleeds for anyone, but I believe—together—we are moving mountains as a global community. Innovation in new treatments is on the horizon, but we must remain strong and vocal in advocating for access to those therapies. Our motto, *Nothing About Us Without Us*, rings truer now more than ever, and I am so proud to be part of this journey with all of you.

Thank you, from the bottom of my heart, for your continued support, your time, your donations, and for simply being part of this incredible family. I can't wait to see you all soon and in March, and throughout the year, as we continue to grow and stand strong together—one voice, one family, one united South Carolina.

With deep gratitude,
Sue, *Executive Director, BDASC*

Volunteers Always Needed, Forever Appreciated

By Erin Van Nostrand

Our BDASC programs are such a success because of our amazing volunteers. We would like to thank each and every one of you for your continued support and contributions. We are grateful that our volunteers return year after year to assist in events that they are passionate about.

We are especially appreciative of our Walk and Golf volunteers! Our annual walk is our largest community fundraiser that helps support the bleeding disorders community throughout the year. Our volunteers are committed to helping raise awareness and funds for our community walk.

Our Par For The Clot Charity Golf Tournament brings in funds from outside corporations and business throughout both South Carolina and the nation. It is critical for BDASC to bring in additional funds to be able to support our community, as industry funds and support continue to decrease while costs continue to rise. This event is hosted by volunteers and committee members who work half the year to ensure it is successful and raises funds to support our robust advocacy work, operations and services we provide, such as camp support, travel grants and scholarships. Speaking of advocacy, we are equally appreciative of our advocacy volunteers who work hard to voice our needs for access to treatment.

You too can become involved in the BDASC community and become a volunteer! Everyone is welcome to volunteer, you do not need a bleeding disorder to become involved and make a difference. If you have a passion for making an impact, we have an opportunity for you! Volunteers are welcomed in the office, along with events working with kids and teens, setting up or handing out items and information. If you would like to volunteer, or would like additional information, please contact Sue at the Chapter office, or register on our volunteer page on our website.

scan for more info

> *"The heart of a volunteer is not measured in size, but by the depth of the commitment to make a difference in the lives of others."*

Help Shape BDASC - Join the Board!

BDASC's mission is to raise awareness for and advocate on behalf of people with bleeding disorders and their families; provide education and supportive services; and promote ongoing research to improve the quality of life for those affected. In order to support this mission, BDASC is comprised of staff, consultants and a Board of Directors. Our Board of Directors are responsible for the governance of the organization and work closely with our staff. The board members and executive director meet monthly to discuss the direction, governance, programs, services, and support for the organization and its members. We accept applications at any time and review as new board positions become available.

This year, we have several members from our Board of Directors that will be leaving the board.We are on the lookout for new members to join our team of dedicated individuals to help guide and shape the organization to meet the needs of our community. If you know of someone who is interested in joining our board, or if you, yourself, are interested, we invite you to learn more at our website, bda-sc.org, or reach out to our Executive Director, Sue Martin, or our BDASC Board President, Aaron Smith.

"Being a part of this community and knowing I have people to lean on when even my own family doesn't understand what I'm going through. It's so amazing to have people what completely understand what it feels like to be in the hospital on Christmas, or to have a port in your chest. I have met people here that have become like family to me and I'm so grateful for them all! I am so thankful Lincoln was born into this community because I never even met anyone with a blood disorder until I was in my 20s and that was really hard. He won't have to experience that, thank goodness, he will always have a place in the Bleeding Disorders Association of South Carolina community." - Taylor Upton

Teen & Parent Outreach - Teens Transitioning to Adulthood

November 30, 2024 - Columbia, SC

T.E.A.M
TEEN TRANSITION, EDUCATION,
ADVOCACY & MENTORING PROGRAM

Driving Their Future

We had an amazing time in Chapin for our first of several program events designed specifically for teens transitioning into adulthood and their parents. This informative "Lunch & Learn," sponsored by Takeda, brought together teens and their families to discuss important aspects of this transition. Our goal was to equip teens with the knowledge and skills needed to navigate their move into adulthood. We covered a variety of topics, including entering the workforce, preparing for job opportunities, and understanding how to explain a bleeding disorder to a potential employer, as well as when it's appropriate to disclose it. We also explored strategies for having open conversations about bleeding disorders with professors, roommates, friends, and even in dating situations. Additionally, we delved into travel considerations—what to pack, how to prepare, and tips for managing a bleeding disorder while traveling. After the event, the group headed over to Frankie's Fun Park for some Go-Cart racing. It was a wonderful opportunity for both teens and parents to bond and enjoy some fun together while continuing to learn. We're excited about upcoming Teen Programming events and look forward to seeing everyone again soon! Be sure to keep an eye out for our next Teen Event!

Takeda

DEDICATION AND PERSONAL SUPPORT

Introducing your Pfizer Patient Affairs Liaison (PAL), a professional dedicated to serving the rare disease community. PALs connect patients, caregivers, and patient advocacy organizations with Pfizer Rare Disease tools and resources. A PAL's work is guided by the principles of **compassion**, **commitment**, and **connection**.

Rachel Cooper-Leal

✉ Rachel.Cooper-Leal@pfizer.com ☎ 910-709-7317

> "I am proud to serve as a trusted resource for the rare disease community"

I'm here to:

Provide compassion by understanding the needs of patients, caregivers, and advocacy organizations. I serve as a point of contact for questions they may have

Maintain commitment by educating patients and caregivers about Pfizer Rare Disease patient support resources

Facilitate connection by providing educational programs that bring patients, caregivers, and advocacy organizations together to share experiences and support their communities

Scan to connect with a PAL
or visit TogetherForRare.com

Pfizer Rare Disease | TOGETHER FOR RARE™

Community Engagements

Virtual Fundraiser: Panda Express

December 3, 2024, Throughout the Country

We want to give a huge thank you to those who were able to participate in our “Day of Giving” Panda Express Virtual Fundraiser! This year, for Giving Tuesday, we hosted a Virtual Fundraiser through Panda Express.This fundraiser allowed anyone, anywhere to head to a Panda Express anytime on Tuesday, December 3rd and place an order to benefit the Bleeding Disorders Association of South Carolina. Panda Express generously donated 28% of the proceeds from our orders to the organization.We were able to raise $58.40 during this fundraiser. Thank you to everyone who supported our organization through this fundraiser!

Winterfest State-Wide Meeting

December 14, 2024 - Columbia, SC

Winterfest Celebration: A Festive Finale to 2024

On a chilly December day, our community came together to celebrate the holiday season and enjoy one last event of 2024 in Columbia! Families were treated to a festive luncheon, complete with amazing entertainment. Guests had the chance to decorate cookies, make crafts, face painting and experience the magic of John Tudor and his bunny, Lucy.

Throughout the event, parents connected with our exhibitor sponsors, learning about the latest treatments and services available to the bleeding disorders community. True to BDASC tradition, the kids enjoyed a heartwarming reading of “The Night Before Christmas,” and were thrilled by a surprise visit from a special guest straight from the North Pole! As part of the festivities, children and teens received special gifts from our North Pole visitor, spreading joy and holiday cheer. Jingles, our beloved BDASC Elf on the Shelf, made his appearance, and the kids had a blast finding him throughout the event. A special Elf on the Shelf package, along with a copy of The Night Before Christmas, was raffled off to delight the little ones.

Thank You Sponsors

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Winterfest was the perfect way to close out the year, celebrating the spirit of the season with our community. During the event, Sue Martin, Executive Director, shared an update on the chapter’s progress and provided important information about what’s to come in 2025. It was a memorable event that truly captured the joy of the holidays, and we look forward to more events in the coming year!





2025 Planning & Sounding Board Meeting

December 17, 2024 @ 7pm - Via Zoom

As we closed out a year of amazing community events, we gathered one last time virtually to discuss what the community would like to see going forward. Ten people attended our Sounding Board meeting to share their input. The conversation centered around programs and support for the upcoming year. Many people suggested we shift some of our focus onto our teens and young adults. It was expressed that it is important to engage teens and young adults before they go out into the world on their own and to show them the importance of our bleeding disorders community. A goal is to also ensure that they know the available resources. Members shared their ideas on how to accomplish this hefty task. Ideas ranged from reaching out to members outside of the community, having classes or support groups for adults at our annual meeting, and having events on transitioning to adulthood. We are happy to report that we have already begun a teen program, called T.E.A.M. to help engage our tweens/teens and beyond. Keep an eye out for more T.E.A.M. events throughout the upcoming year.

VWD, Rare & Platelet Reach & Teach Virtual Event

January 21, 2025 - Via Zoom

During our annual Fall Family Advocacy Camp, it was requested by the VWD and Rare communities to have virtual events dedicated to the unique challenges these two communities face. We heard your request and made it happen with our Virtual Reach & Teach! We have scheduled quarterly zoom meetings for our VWD, Platelet Disorders and Ultra Rare communities to gather together virtually to share updates on the VWD, Platelet & Ultra Rare communities and to support each other. Our first meeting was held on January 21st and was a success. While we did not have a large attendance for this first meeting, what we lacked in numbers we made up for in support. We had community members from all over the state join us and connect. Be sure to save the date for our next Virtual Reach & Teach on **May 20, 2025.**

T.E.A.M. Teen Transitioning Event

February 8, 2025 - Columbia, SC

Our time together in Columbia was a celebration of the collective strength and determination of our community. Teens, tweens, and their families joined forces to support our New T.E.A.M. Program, sponsored by CSL Behring. Tweens and teens participated in engaging educational activities like the game of Rolling the Dice in Life where they rolled big dice on a game board and where they landed, they had challenge and discussion cards they answered with the group. After a fun lunch where parents also had time to share and connect, a collaborative team-building exercises at Saluda Shoals Park was enjoyed by the participants where together, tweens and teens, received inspiring leadership development. As we look to the future, we're confident that these emerging leaders will be the driving force behind our Bleeding Disorders Association of South Carolina Chapter's continued growth and success. We continue to build upon our new T.E.A.M. Program, so be sure to check out our teen support webpage for more information and upcoming events. Our next event is scheduled for **May in the Lowcountry**. More information will be available soon.

scan for teen
transition guide

CSL Behring
Biotherapies for Life™

T.E.A.M
TEEN TRANSITION, EDUCATION,
ADVOCACY & MENTORING PROGRAM

Upcoming Community Engagements

VWD Awareness Day Virtual Event

March 1, 2025 - Via Zoom

Join Us for the National Bleeding Disorders Awareness Month: Virtual Event on March 1st

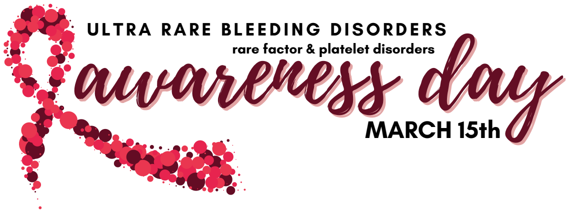
This virtual gathering is designed to explore and learn as we raise awareness and celebrate our connections to the von Willebrand Disease Community during our second annual von Willebrand Disease Awareness Day on March 1st! We're kicking off our series of awareness days by hearing from Morgan Cook, sponsored by Takeda. Our conversation will be centered around– "Resilience." We invite our community to come share their stories, learn, and connect, and let us all raise awareness for VWD during the National Bleeding Disorders Awareness Month of March! This celebration is for everyone in the community. Register for our virtual event here and on our website.

Scan To Register

Takeda

Ultra Rare Bleeding Disorders Awareness Day Virtual Event

March 15, 2025 - Via Zoom



Join Us for National Bleeding Disorders Awareness Month: Virtual Event on March 15th

On March 15th, we'll come together virtually to raise awareness and show support for our Ultra Rare Bleeding Disorders Community and Platelet Disorders members during National Bleeding Disorders Awareness Month.

We invite all rare and platelet disorder community members to join us for this special day where you can share your stories and connect with others. Don't miss this opportunity to unite, raise awareness, and celebrate our community. Register for our virtual event here and on our website!

Scan To Register

"Go Red" for St. Patrick's Day Event

March 17, 2025 - Braza Do Sul Brazilian Steakhouse 410 Columbiana Dr, Columbia, SC

Go Red on St. Patrick's Day

Join Us for National Bleeding Disorders Awareness Month

We are "Going Red" on March 17th in Columbia for a Community Engagement Dinner to learn about Positive Self-Talk! Our educational conversation will revolved around Positive Self-Talk. We will explore behavioral insights that may help you discuss your bleeding disorders management with your loved ones and healthcare team.

Be sure to wear Red & Green to celebrate both Bleeding Disorders Awareness and St. Patrick's Day! We chose March 17th for our "Go RED" day to help start conversations around why we are wearing Red on a day where you are supposed to wear Green!

sanofi

Upcoming Community Engagements

Women Who Bleed Awareness Day Virtual Event

March 30, 2025 - Via Zoom

Join Us for National Bleeding Disorders Awareness Month: Virtual Event on March 30th

This virtual gathering is designed to explore and learn as we raise awareness and celebrate our connections to the Women Who Bleed Community during our first ever Women Who Bleed Awareness Day on March 30th! We're rounding out our series of virtual awareness days by hearing from Brittany Savage, Nurse Practitioner, sponsored by Novo Nordisk. Our conversation will be centered around– "Women With Hemophilia." We invite our community to come share their stories, learn, and connect, and let us all raise awareness for Women Who Bleed during the National Bleeding Disorders Awareness Month of March! This celebration is for everyone in the community. Register for our virtual event here and on our website.

Scan To Register

Adult Connections Retreat

April 4- 6, 2025 - AC Hotel Greenville 315 S Main St, Greenville, SC

We're thrilled to invite our adult members, ages 18 and up (with an adult 21+), who are living with bleeding disorders, are carriers of a genetic bleeding disorder, or are caretakers of someone affected by a bleeding disorder to come together for a truly special weekend! This event promises to be a one-of-a-kind experience where you'll connect with others who share your journey, recharge, and make lasting memories.

Whether you're looking for a refreshing break from daily responsibilities, an opportunity to meet new friends, or a chance to strengthen the bond with your partner, this weekend is for YOU! Parents can relax and recharge, while single men and women have the chance to connect, share experiences, and support one another. Together, we'll celebrate our shared strength. We will also have educational sessions designed to support our von Willebrand community.

Evening Entertainment at:

Join us for Caregivers Compass where experiential education meets heartfelt conversations

Learn More & Register Here

World Hemophilia Day Celebration

April 17, 2025 - Grand Bohemian Lodge, 44 E Camperdown Way, Greenville, SC

Join Us for National Bleeding Disorders Awareness Month: Final Event on April 17th

Join us as we celebrate World Hemophilia Day - Recognizing All Bleeding Disorders. This year's event theme is "Women & Girls Bleed Too." We will enjoy dinner, activities, and community connections, sponsored by Sanofi.

Scan To Register

Upcoming Community Engagements

STEP for bleeding disorders

5K WALK/RUN

Saturday, May 10th @ Saluda Shoals Park, Columbia

JOIN US!

Learn more and register at https://tinyurl.com/2025STEP

H.E.L.L.O Annual Meeting & Bleeding Disorders Conference

June 6-7, 2025 - Embassy Suites Golf and Conference Center by Hilton 670 Verdae Blvd., Greenville, SC

Join us as we come together in unity and EMBRACE the opportunities of learning, connecting, sharing, and networking within the bleeding disorders community. Hear from experts in the field of bleeding disorders care and treatment. There is no other event like this one in our state. You will leave feeling empowered with support and knowledge. Please join us and see what it is all about. Please be sure to read all the information about the conference on our website page and apply online. We can't wait to be together again!

Friday Night Opening Annual Meeting

Join GutMonkey and BDASC for an interactive and engaging opening session that will have you up, moving, and connecting with community members. During the General Session for the Leading Edge program, we'll focus on kicking off our time together for the weekend through games and connections. We'll reflect on the power of gathering and set intentions for the weekend. We will explore these ideas using games and community building activities that will have participants on their feet and interacting with each other in fun and memorable ways. This session is part of the Leading Edge program and is made possible through the exclusive support of Pfizer.

GUT MONKEY

2024 Award Recipient, Warren Ingram

Friday Night Chapter Awards

Join BDASC in celebrating out Chapter Award Recipients! Annually, BDASC chooses a selecting of outstanding individuals who embody the mission of the chapter. Chapter awards include Board Member of the Year, Legislative Advocate of the Year, Junior Advocacy Ambassador of the Year, the Volunteer of the Year and more. Academic scholarships are awarded to those members who are attending or plan on attending institutes of higher education in the fall. These scholarship recipients have applied through BDASC's Academic Scholarship program.

Registration applications for the 2025 HELLO Annual Meeting & Bleeding Disorders Conference will be available in March. Please be sure to visit out website to learn more.

Learn More Here

HEMGENIX®
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Want to hear from someone who has received HEMGENIX?

Watch Michael's Story here:

Visit HEMGENIX.com to learn more

— Michael, 23-year-old treated with HEMGENIX

Actual HEMGENIX patient. Patient experiences may vary.

CSL Behring

USA-HGX-0810-AUG24

Voices of Our Community

your voice matters

This issue of The News Infusion includes a new version of the Voices of our Community. We have enjoyed reaching out to community members asking them to share their voices. We have been on a mission to ensure that every member of the bleeding disorders community is represented and heard. With the implementation of the Voices of 46 and providing our community members a microphone in which to share their stories, we hope to encourage every BDASC member to advocate for themselves and share their experiences. To learn more about our Voices of 46 program, visit our website at www.bda-sc.org or attend one of our outreach program events.

Would you like to be featured in our "Voices of Our Community" section of our newsletter? We would love to share your voice. Please reach out to the Chapter Office at 864-350-9941 or email Sue Martin at sue.martin@bda-sc.org.

Strengthening Partnerships to Support Our Community

By Sue Martin

The relationship between our Chapter and Treatment Centers is built on strong, cooperative partnerships aimed at addressing the needs of the South Carolina Bleeding Disorders Community. We meet several times each year to discuss updates, community needs, and any concerns or suggestions from our members.

These collaborative meetings have led to the development of key initiatives, including teen transition programming, infusion clinics and training sessions, and outreach efforts to better serve our rural communities. We are also addressing concerns related to rural hospitals, emergency room care, and urgent care facilities. One of our key projects includes the creation of a new emergency room brochure, which will be distributed to our members once completed. This resource will help empower our community with a voice in treatment and care needs.

In addition to these initiatives, our partnership also focuses on addressing the travel and financial needs of our patients and members. By working together, we are able to share resources and provide essential support. Our Patient Advisory Committee members consists of patients of our HTCs, staff from BDASC, and our HTC physicians and staff. We look forward to continuing this partnership throughout the years ahead.

BDASC -South Carolina Treatment Center Information

Prisma Health - Hemostasis and Thrombosis Center of South Carolina

MUSC Pediatric Hematology/Oncology

We all have a story and each one of us can make a difference for the community. Meet Joshua, one of our young adult ambassadors for Bleeding Disorder Association of South Carolina.

My ITP

By Joshua P. Douglas

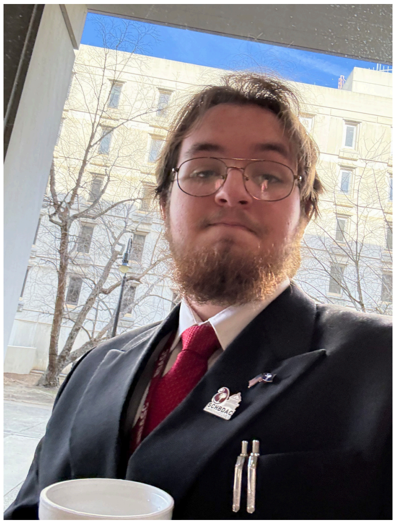
Despite my work as editor for my school's paper, I don't really know how to write an article like this.

First, I guess I should introduce myself. I don't have Hemophilia. In fact, I don't even have a family history of bleeding or any genetic markers for my bleeding disorder. Because Immune Thrombocytopenic Purpura (ITP) is not a hereditary illness. ITP is more so an injury. It's a faulty immune response that can be caused by viral infections like Measles, Long Covid, or the Mumps, or by the adverse effects of certain vaccines.

In my case, all signs indicate that my Chronic ITP developed when I received the MMR Vaccine. This, compounded with certain immune effects of my Ehlers Danlos Syndrome, managed to trigger the otherwise extremely unlikely course of events that can cause a 4-year-old child's immune system to begin destroying healthy platelets.

14 years and countless prayers later, there is still no easy treatment available for me. The only option I have to boost my platelet count is to be hospitalized and given an infusion overnight whenever my doctors and insurance agree that it's absolutely necessary. So, when I saw that there are medications that work for people with other bleeding disorders and then saw insurance companies trying to shortchange them and needlessly deprive them of those treatments, I was livid. I knew I had to help however I could.

Thankfully, I've always been interested in politics. I've had the opportunity to attend 6 separate youth conferences for model government, where I learned how to advocate for myself and others in this community. I've also seen just how important it is that we speak up for ourselves and for each other, because we all have different stories to tell.



Emergency Preparedness

By Samantha Javorka

Living with a bleeding disorder or caring for someone with a bleeding disorder can lead to many challenges. Families live with different expectations and different life experiences which greatly shape their lives. Being prepared for the unexpected is even more necessary.

Here at the Bleeding Disorders Association of South Carolina, we aim to ensure that our member families have the resources needed to best prepare them for the unexpected. We will be hosting an event for our young and new families this spring titled “Expect the Unexpected.” This event will provide insight into how you can be prepared for emergencies and how best to handle these situations. We are currently working on creating a brochure for each of our members to be able to bring to the Emergency Room to ensure that they are taken seriously – or so they can best advocate for themselves during stressful situations.

We meet with our HTCs on a regular basis to ensure that they are aware of barriers to care that their patients have expressed to us. Because of this, we’ve been able to allow our HTCs to provide information on our medical alert bracelet program. The HTCs have also implemented “Medical Cards” – which provide patients with a card explaining their diagnosis and what medical professionals should do in an emergency. The HTCs also provide “Travel Letters” – which are more detailed explanations of the patients bleeding disorders and provide contact information for their HTC. These “Travel Letters” can be used for several things – providing information to schools, new physicians, emergency rooms, and allowing you to bring medication on planes and more.

BDASC Provides New Emergency Room Resource Brochure

Another way that BDASC is helping to provide our community members with important resources is by creating a series of brochures. These brochures are for community members to be able to advocate for themselves. We already have brochures for school children - resources for their teacher, their parents & the school nurse, and college students. We are thrilled to be able to share our new brochure with the community. This brochure is for use when visiting an Emergency Department. There is a space on each brochure for each patient to provide necessary information for the Physicians to be able to treat them. This brochure also provides contact information for each of our treatment centers throughout the state. Download a copy or reach out to the Chapter Office (864-350-9941 or info@bda-sc.org) to receive a physical copy. These copies are also available at BDASC events throughout the year.

NBDF HANDI

MASC ER Information

Family Emergency Checklist

STEPS For Living

Ready.Gov

Treatment Center Info

BDASC Mental Health Resources

How to Prepare for an Emergency

1. Know you or your family member's **diagnosis and treatment**. Be able to provide this information even when stressed. For small children, have them practice their diagnosis and have them repeat it to you.
2. When possible, **have medications on hand**.
    a. Know when to order more and how to do this.
    b. During extreme weather conditions, be sure to have extra doses on hand. Ensure that you have a back-up plan in case of power outages.
3. Where a **medical alert bracelet/necklace**. Your medical alert should contain your full name, your diagnosis and the HTCs phone number.
4. Know the location of your nearest Emergency Department.
    a. Not all emergency departments can treat patients with bleeding disorders.It may be necessary to call ahead to determine if your closest emergency department has your product available to them or if they will direct you to another hospital.
    b. You may be able to bring your medication with you to your Emergency Department. Call ahead to determine if they allow you to do this.
5. Bring your **medication, travel letter and medical card** with you to the Emergency Department.
6. **Know your HTC Contact information** (store this information in your phone). Before arriving at the emergency department, if you are able, call your HTC directly. Let them know that you are having an emergency and which hospital you are going to. They can then call ahead to the hospital to let them know you are coming and what they need to do.
    a. If you have not called your HTC directly, you may have to ask for the Emergency Department staff to do so.
7. **Remember: You are an expert at your bleeding disorder.** Bleeding disorders are rare and complex. You are your best advocate for yourself. Do not be surprised if Emergency Department staff are unfamiliar with your bleeding disorder.

BDASC Provides Medical Alert Resources

Bleeding Disorders of South Carolina believes one way to protect yourself or your kids with bleeding disorders is to be sure a Medical Alert ID is worn at all times. The information you provide on these IDs will help the emergency medical providers and medical teams identify your condition and medical needs should you not be able to speak for yourself. These ID's can very well save your life. Medical IDs come in Rugged Sports bands to Pedant Necklaces and are more than just a piece of jewelry; they provide you with constant access to the world's largest emergency-support network.

Bleeding Disorders Association of South Carolina can provide assistance to help purchase a Medical Alert ID for South Carolina's Bleeding Disorders registered members of the Chapter. Please contact the office for additional detailed information. We have provided a few resources on our website, however, you may obtain your Medical Alert ID from many additional resources that are available to you. If you would like to request financial assistance to purchase your Medical Alert ID, please submit the financial assistance application form by downloading the application and sending it into the office by email or mail, or you may apply using the online application. The Chapter will contact you once your application is approved to work with you to get the Medical ID ordered and shipped to you directly. Make an application request if you need assistance. We are here to help!

BDASC Medical Alert ID Resources

Investigational VWD Therapy to Receive Expedited Review from FDA

The U.S. Food and Drug Administration (FDA) has granted Fast Track designation to the investigational von Willebrand disease (VWD) therapy VGA039. The therapy is being developed by Star Therapeutics, a biotechnology company based in San Francisco, CA.

VGA039 is a subcutaneously-delivered monoclonal antibody therapy designed to target Protein S as a means of restoring proper blood clotting in people with all types of VWD. An important regulator of clotting within the coagulation cascade, protein S works with other proteins in the body to prevent the formation of excessive blood clots.

Subcutaneous (Sub-Q) therapies require an injection just under the skin, as opposed to intravenously administered products which necessitate venous access. As an easier to administer therapy, Sub-Q treatments have the advantage of greater convenience which in turn may increase rates of adherence.

The new fast track designation follows recent reporting of positive interim clinical trial data for VGA039, which was presented during the 66th American Society of Hematology meeting in December 2024. FDA's Fast Track designation is meant to facilitate the development and expedite the review of drugs that treat a broad range of serious conditions and address an unmet medical need. The purpose is to get newer drugs in clinical development through FDA's approval process faster and ultimately to the patient earlier.

If VGA039 should receive FDA approval, it would be the first Sub-Q therapy to be available to treat all types of VWD.

"VGA039 is the first drug candidate to receive Fast Track designation for VWD, and we are committed to advancing drug innovation for this debilitating disease that has lagged behind other bleeding disorders," said Adam Rosenthal, PhD, CEO and Founder of Star Therapeutics. "Fast Track designation enables us to potentially accelerate the development path for VGA039 as a therapy that can transform the way VWD is treated with a convenient, subcutaneous therapy for patients with all types of VWD."

Source: Businesswire, January 8, 2025
Printed with permission from the National Bleeding Disorders Foundation

Learn more about VGA039 and Star Therapeutics

Learn more about Fast Track criteria and processes on the FDA website.

Hemab Therapeutics Presents Interim Data from Ongoing Phase 2 Study of HMB-001, First Ever Prophylactic Treatment in Glanzmann Thrombasthenia & Preclinical Data from HMB-002 in VWD at the 2025 EAHAD Annual Congress

COPENHAGEN, DENMARK AND CAMBRIDGE, MASS., US – February 7, 2025 – Hemab Therapeutics, a clinical-stage biotechnology company developing novel prophylactic therapeutics for serious, underserved bleeding and thrombotic disorders, today presented interim data from the ongoing evaluation of HMB-001, a novel bispecific antibody in development as first ever prophylactic treatment for the bleeding disorder Glanzmann thrombasthenia (GT). The Phase 2 study consists of a minimum 6-week prospective run-in where participants record bleeds via an electronic bleed diary, followed by 3 months of treatment with HMB-001. Interim efficacy data to date demonstrated >50% reduction in treated bleeds in all 3 tested dose cohorts. Underscoring its potential to address unmet medical needs and to expedite patient access, HMB-001 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration and the UK Medicines and Healthcare products Regulatory Agency has awarded it designation under the Innovative Licensing and Access Pathway.

Hemab also unveiled compelling preclinical data for HMB-002, a potential novel treatment for Von Willebrand Disease (VWD). These data were featured as oral presentations at the 18th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) held this week in Milan, Italy. "Glanzmann thrombasthenia is a serious and devastating bleeding disorder where patients often experience in excess of 60-80 treatment-requiring bleeds annually. These interim Phase 2 results signal the potential of a new reality for the Glanzmann thrombasthenia community, bringing us a step closer to a much-needed breakthrough in preventing bleeding," said Kate Madigan, Chief Medical Officer at Hemab. "The preclinical data for HMB-002 highlights its potential to specifically address the underlying patho-etiology and revolutionize care for the Von Willebrand Disease community, offering hope to prevent life-threatening complications and provide patients with a critical new lifeline."

Interim pharmacokinetic (PK) data from the Phase 1/2 study of HMB-001 supports the use of weekly or biweekly dosing regimens. Pharmacodynamic (PD) responses are aligned with the mechanism of action,demonstrating a dose-dependent accumulation of activated Factor VII (FVIIa) and enhanced potentiation through TLT-1 binding. Efficacy assessments indicate a reduction of over 50% in treated bleeding episodes at all tested dose levels.

Read the Full Press Release Here

With Gratitude and Heartfelt Appreciation, We Thank Our Community Partners and Donors

Words cannot fully express our gratitude to our donors. Your belief in our cause empowers us to continue our work and create positive change for those living with bleeding disorders in South Carolina.

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BDASC Legislative Breakfast, Another Success

by James Romano, BDASC Legislative Advocacy Consultant and Coordinator

The Bleeding Disorders Association of South Carolina (BDASC) has a long-standing history of collaboration with the South Carolina Legislature, but recently, thanks to your involvement, we've been able to significantly broaden our reach, working with an increasing number of members from both the South Carolina House of Representatives and the Senate. A key strategy in expanding and strengthening these relationships has been our annual Legislative Breakfast, hosted each January. This event has proven to be an invaluable opportunity to foster connections, engage directly with lawmakers, and continue advocating for the needs of individuals affected by bleeding disorders. Through this consistent presence at the Capitol, BDASC is reinforcing its role as a trusted partner in advancing meaningful legislative change.

This year, BDASC proudly hosted its 3rd Annual Legislative Breakfast at the Capitol on Wednesday, January 15th, 2025. This event provides an invaluable opportunity for Chapter leadership and patient advocates to engage directly with members of the legislature and Capitol staff in a relaxed, informal setting over a delicious breakfast. Averaging around 250 attendees each year, this year's event was no exception in its success. Catered by Lizard's Thicket, the morning allowed our members to connect with lawmakers and discuss the community's legislative priorities in a meaningful and personal way. The breakfast continues to be a key event in strengthening our advocacy efforts and building lasting relationships with those who can make a real difference.

This year, BDASC added a special touch to the Legislative Breakfast with a brief but impactful program. Attendees were honored to hear from Representative Carla Schuessler (R-61st), one of our dedicated community champions, who shared her continued support for our cause. Following Representative Schuessler's remarks, two patient advocates, Virginia Maxwell and Colt Gallaway, spoke candidly about their personal struggles navigating health insurance with copay accumulators. Their powerful testimonies brought the real-world challenges faced by our community to the forefront. After the breakfast, our members took the opportunity to personally visit their legislators, continuing the important conversations and advocating for meaningful change.

When Representatives Hardee (R-105th) and Schuessler (R-61st) introduced our Copay Accumulator bill (H. 3934), it was with the support of approximately 70 cosponsors. This level of backing reflects the success of our advocacy efforts, and the BDASC Legislative Breakfast played a crucial role in generating early momentum for our legislative agenda. Your involvement—and that of your family—was instrumental in making this progress possible. As we look ahead, we're excited to continue this work together and look forward to seeing you on BDASC Advocacy Day later this month, where we'll continue pushing for the change our community needs.

IMPORTANT ADVOCACY DATES

Feb. 25 & 26: BDASC Legislative Days, Columbia
Mar. 5 - 7: NBDF Washington Days, Washington, DC
March 1: Von Willebrand Disease Awareness Day
March 15: Ultra Rare Bleeding Disorders Day
March 30: Women with Bleeding Disorders Day
April 17: World Hemophilia Day

Learn more about H.3934 and Senate Bill S.330

SCHBDAC
The South Carolina Hemophilia and Bleeding Disorders Advocacy Coalition

State Advocacy Days at the Capitol

February 25 - 26, 2025, Columbia, SC

Our Annual Advocacy and State Legislative Days helps kick off the National Bleeding Disorder Awareness Month. Our event is held in Columbia, SC at the Capitol Building. BDASC, along with our national partners, host advocacy training sessions 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. Advocates have the opportunity to have important discussions and to ask for support on key areas of concern for access to affordable treatment care. Since its inception, our State Capitol Advocacy Days have provided for over 637 individual meetings with the SC House of Representatives and our State Senators from districts throughout the state. These meetings are of key importance in helping our elected officials understand our communities' unique needs, how access to medications and treatment are critical, and asking for continued 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 our advocates can help remove these barriers with their advocacy participation! This advocacy event is for the whole family with teen advocacy programming included. We are grateful to all who participate. Thank you for raising your voice!

Participants in State Advocacy Days are requested to join BDASC for a Pre-Advocacy Days virtual training webinar. This training session helps guide our advocates and provide them with the necessary tools to have a successful State Advocacy Day. A full wrap up report will be provided in the next issue of the News Infusion.

Advocacy training is provided in partnership with our national organizations and the South Carolina Hemophilia and Bleeding Disorders Advocacy Coalition and Ambassadors; The National Bleeding Disorders Foundation, Hemophilia Federation of America, our stakeholders, and other local and national advocacy partners.

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A Message from the Heart of NBDF

January 28th, 2025

NBDF wants to reaffirm our unwavering commitment to our entire bleeding disorders community. Our mission—enabling every person and family affected by bleeding disorders to thrive—remains unchanged. We stand resolute in our dedication to strengthening equitable healthcare access across our diverse nation, ensuring no one faces their bleeding disorder journey alone.

To our community members including marginalized and minoritized populations, women, immigrant families, persons with disabilities, transgender, LGBTQIA, rural and frontier populations, low income, low literacy, English learner, and all who face healthcare barriers: we see you, we hear you, and we stand with you. Your experiences shape our work and drive our mission forward.

Health equity means meeting people and communities where they are and providing what they need to become healthier within the environment where they live, work, worship, play and learn. Within health equity, we must recognize that people and communities are diverse in their experiences and backgrounds.

NBDF is deeply committed to fostering diversity, equity, inclusion, belonging, and access (DEIBA), promoting health equity, and combating racism, homophobia, xenophobia and classism. We will continue our Health Equity, Diversity, and Inclusion (HEDI) strategic approaches and priority population efforts.

We also recognize the unique challenges faced by minority and marginalized populations and are committed to addressing these through comprehensive education, robust advocacy, impactful research and programming.
In partnership with community members, healthcare partners and industry stakeholders we will continue to:

- Amplify our Lived Experience Experts' (LEEs) voices
- Promote health equity, diversity and inclusion at NBDF
- Recognize and support Women, Girls and People with the Potential to Menstruate (WGPPM)
- Strengthen support for underserved communities
- Advocate for inclusive policies that serve all community members

Our hope is to build a future where every person with a bleeding disorder can access quality care and live life to the fullest. Our ongoing efforts are focused on ensuring that all individuals, regardless of their background, have access to the resources and support they need. NBDF stands firmly in its belief that healthcare decisions must be between a person/family and their healthcare providers. We look forward to continued collaboration with all stakeholders in pursuit of our shared goals for equitable healthcare access.

Together, we can create a more inclusive and equitable future for all.

Learn more about how the foundation partners with stakeholders to reduce burdens, meet unmet patient needs, and improve health equity.

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[*] Once well-controlled (1 month without spontaneous bleeding or requiring dose adjustments on a weekly dose of ≤40 IU/kg), people 12 years and older can be transitioned to 14-day dosing.

[†] The average dose for adolescents and adults receiving prophylaxis every 7 days was 37 IU/kg.

[‡] The median AsBR for people who started on 7- or 14-day prophylaxis was 0. For people who switched to prophylaxis from on-demand, the median AsBR was 0.7.

AsBR=annualized spontaneous bleed rate.

IMPORTANT SAFETY INFORMATION

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Stop treatment and immediately contact your healthcare provider if you see signs of an allergic reaction, including a rash or hives, itching, tightness of chest or throat, difficulty breathing, lightheadedness, dizziness, nausea, or a decrease in blood pressure.

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TO LEARN MORE, GO TO **WWW.IDELVION.COM**

CSL Behring

HTC Looks at Family Pedigrees and Hemophilia Carrier Screening

December 5, 2024

A new study published in the journal Blood Advances describes a new initiative focused on traditionally underserved and underdiagnosed subgroup within the bleeding disorders community – hemophilia carriers/females with hemophilia.

The study was spearheaded by a hemophilia treatment center (HTC) at the Cliniques universitaires Saint-Luc, in Brussels, Belgium. While the HTC has been screening female relatives of patients with hemophilia for two decades, accurate information on all carriers/potential carriers within the families of their patients was lacking. The investigators therefore set out to identify potential and obligate hemophilia A (HA) and hemophilia (HB) carriers by updating the family pedigrees of all people with hemophilia (PWH) receiving regular care at the center. Several scenarios denote obligate carrier status, such as being the daughter of a man with hemophilia.

The study was conducted at the HTC from May 2021 to April 2023. A retrospective data analysis of electronic medical records generated from routine carrier screening of female relatives of PWH. The analyses captured data on factor VIII and factor FIX levels, genetic testing results, bleeding history, hemostatic treatment history, and family pedigrees.
Investigators also took the opportunity to identify family members that were previously unaccounted for. “In addition to this retrospective analysis and to further enhance the screening of female relatives of PWHs, we adopted a proactive and systematic approach to reach out to previously unscreened or incompletely screened individuals, explained the authors. “During personal contacts with PWHs regularly followed at the HTC (referred to as “probands” hereafter), either during clinical follow-up visits or telephone interviews, pedigrees of probands were analyzed and unscreened potential and obligate carriers of hemophilia were identified, complementing the pedigree information gathered during the retrospective part of the study.”

The results showed that relatively large groups of carriers within the HTC’s sphere were previously identified, with some omissions. In pedigrees of 287 male PWHs (226 HA and 61 HB) and seven female patients from 236 families (184 HA and 52 HB), a total of 900 female individuals were identified. Of those, 454 were obligate and/or genetically proven carriers, and 118 were noncarriers.

Subsequent genetic testing was conducted in 133 obligate, 237 potential, and four sporadic carriers, with 190 obligate and 328 potential carriers yet-to-be tested. Among carriers with known factor levels (261/454), 42 HA (23.0%) and 23 HB carriers (29.5%) had a factor level below 40 IU/dL, suggesting mild hemophilia. Carriers with a factor deficiency were screened on average six years earlier than other females.

The investigators emphasized the importance of robust efforts in screening for potential hemophilia carriers to ensure that less individuals fall through the gaps.

“The imperative to continually update pedigrees remains essential, given that even groundbreaking treatments such as gene therapy do not alter the hereditary nature of hemophilia. In addition, the transition of PWHs from pediatric to adult care marks a vulnerable phase during which keeping track of the patient's relatives becomes challenging,” assert the authors. “Furthermore, girls who underwent coagulation factor level assessments during childhood still require genetic testing later in life and should not be overlooked. Identified carriers, especially those experiencing abnormal bleeding, should benefit from regular follow-up at the HTC.”

The paper also cited several limitations, including the retrospective quality of the data, certain selection bias, and lack of insight into patients' factor levels. For instance, only the lowest factor levels of each participant were included in the analysis, no adjustments were made for the use estrogen and/or progesterone treatments, and factor levels measured during pregnancy were not considered.
Limitations notwithstanding the authors posit that initiatives such as these could help further the cause of gender equity in bleeding disorders care.

“To conclude, this study, to our knowledge, represents the first reported initiative to systematically identify and screen (potential) hemophilia carriers among families of all PWHs followed at a single HTC. We have gained new insights into practical aspects of carrier diagnostics, while also identifying persistent challenges in providing comprehensive screening to relatives of PWHs,” explained the authors. “Initiatives similar to our study should be replicated by other HTCs internationally, given that they would represent a crucial step toward ensuring equitable access to hemophilia diagnosis and care for all potentially affected individuals, regardless of sex and gender.”

Read the full open access article to learn more about the study’s findings, including the challenges associated with broader carrier screening, plus insights on symptoms and treatment of these populations.

Citation
Krumb E, Lambert C, Van Damme A, Hermans C. Proactive systematic hemophilia carrier screening: a step toward gender equity in hemophilia care. Blood Adv. 2024 Oct 22;8(20):5268-5278. doi: 10.1182/bloodadvances.2024013866. PMID: 39167764; PMCID: PMC11492442.

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Source: https://www.bleeding.org/news/htc-looks-at-family-pedigrees-and-hemophilia-carrier-screening

In single-ascending dose study in VWD patients, a subcutaneous dose of VGA039 was associated with substantial reductions in bleeds

Star Therapeutics

Additional ASH posters show real-world patient data that identify over 50,000 diagnosed and treated VWD patients in the US and highlight high disease and treatment burden with current standard of care

SOUTH SAN FRANCISCO, CA, December 9, 2024 – Star Therapeutics, a clinical stage biotechnology company discovering and developing best-in-class antibodies, today announced interim clinical data from VIVID 2 (VGA039 Investigation in Von Willebrand Disease), a Phase 1 single ascending dose (SAD) study of VGA039 in Von Willebrand Disease (VWD) patients. VGA039 is an investigational monoclonal antibody that targets Protein S to restore balance in blood clotting, as a universal hemostatic therapy for numerous bleeding disorders. It is potentially the first subcutaneous therapy that addresses all types of VWD and has a convenient dosing regimen. The VGA039 clinical data is being presented at the annual meeting of the American Society of Hematology (ASH) on December 7-10, in San Diego, California.

The interim clinical data in VIVID 2 from three VWD patients with high bleeding rates showed that a subcutaneous dose of VGA039 was associated with substantial reductions in annualized bleed rate (ABR) in line with currently approved VWD prophylaxis therapies. In the interim VIVID 2 data, VGA039 sustained multi-week therapeutic concentrations following a single subcutaneous dose, in contrast to currently approved VWD prophylaxis therapies that require multiple intravenous (IV) infusions every week. These VWD patients had high bleeding rates with ABRs greater than 50 bleeds per year.

“Drug innovation for VWD has lagged behind advancements in treatments for other bleeding disorders, like hemophilia. VGA039 offers a promising breakthrough as a subcutaneous therapy, potentially transforming care for VWD patients by reducing their high treatment burden,” said Dr. Nicholetta Machin of the Hemophilia Center of Western Pennsylvania and University of Pittsburgh. “The interim clinical data is particularly encouraging, showing significant bleed reduction in VWD patients with high baseline bleeding rates.”
Interim clinical data from VIVID 2 (Phase 1 SAD study in VWD patients)

The interim clinical data from VIVID 2 reported in the poster presentation at ASH 2024 describe results in VWD patients who received a 3.0 or 4.5 mg/kg dose of VGA039 administered subcutaneously and evaluated over 8 weeks, including the following highlights:

- In the seven VWD patients evaluated for safety, pharmacodynamic (PD) and pharmacokinetic (PK) activity, VGA039 was safe and well-tolerated with no injection site reactions observed. VGA039 demonstrated pharmacokinetics and pharmacodynamics that support convenient, prophylactic subcutaneous dosing.
  - Therapeutic concentrations of VGA039 were sustained for approximately 4 weeks following a single subcutaneous 4.5 mg/kg dose.
  - VGA039 showed PD activity across all types of VWD patients, including Type 1, Type 2 and Type 3.
- In three VWD patients (a Type 2M and two Type 3) with high historic bleeding rates, a single subcutaneous 4.5 mg/kg dose of VGA039 resulted in 75% to 88% reductions in annualized bleeding rate (ABR). These reductions are consistent with those observed in clinical trials of approved VWD prophylactic therapies that require multiple IV infusions every week.

“These initial results showing a significant reduction of bleeds in VWD patients highlight the exciting prospects ahead for VGA039. We are keenly aware of the unmet need of VWD patients and are working to bring the first potential subcutaneous therapy for patients with all types of VWD,” said Gary Patou, MD, Chief Medical Officer of Star Therapeutics. “Based on the positive results, we are moving ahead with our plans to begin a multi-dose study with VGA039 in early 2025.”
Additional ASH posters and session details
Star Therapeutics is also presenting three other posters at ASH 2024, including two posters with analyses of large real-world VWD patient data sets highlighting the significant disease burden and unmet treatment need in VWD.

- The posters describe data from more than 50,000 diagnosed and treated VWD patients in a large US claims dataset.
- VWD patients experienced high disease burden with bleeding comorbidities, including anemia, heavy menstrual bleeding, nosebleeds, gastrointestinal bleeds, and joint bleeds, sometimes requiring blood transfusions.
- There still remains a disparity in VWD treatment with low prophylaxis use, likely due to the high treatment burden of currently available therapies, supporting the need for less frequent treatment options for VWD patients.

About the VIVID Clinical Program
The VIVID multinational clinical program is a platform multi-phase protocol for the development of VGA039 in Von Willebrand disease (VWD). Patient bleeding is quantified by the number of bleeding events per year or annualized bleeding rate (ABR). For the Phase 1 study, VIVID 2 is the single ascending dose study in VWD patients (NCT05776069).

Resources and Contact Information

Medical Facilities

Prisma Health Hemostasis and Thrombosis Center of SC - Midstate

For all new and previously scheduled appointments, factor refills, school needs, general questions or general concerns: Contact Robin Jones, MSN, MHA, RN, CPN, CPHON, Nurse Navigator, SC Hemophilia Treatment Center at 803-434-1028 or email: schemophilia@prismahealth.org.

Midlands
14 Richland Medical Park Rd., Suite 410
Columbia, SC 29203
Phone: 803-434-3533

Florence
101 William H Johnson Street, Suite 400
Florence, SC 29506

Orangeburg
1724 Village Park Drive
Orangeburg, SC 29118

Prisma Health Hemostasis and Thrombosis Center of SC - Upstate

MAIN CAMPUS:
BI-LO Charities Children's Cancer Center Serving Hematology/Oncology Patients
900 W. Faris Road
Greenville, SC 29605
Phone: 864-455-8898
Fax: 864-455-5164
Hours: Mon, Wed., Thurs. 8:00-4:30;
Tues & Fri. 8:00-12:00

SATELLITE OFFICE:
Spartanburg- Children's Hospital Outpatient Specialties
1700 Skylyn Drive, Suite 200
Spartanburg, SC 29307
Phone: 864-716-6490
Fax: 864-596-5164
Hours: Wednesday - alternating mornings and afternoons

SATELLITE OFFICE:
Anderson - Pediatric Specialties of the Upstate
2000 E. Greenville Street,
Suite 3500
Anderson, SC 29621
Phone: 864-716-6490
Fax: 864-716-6492

Medical University of South Carolina Shawn Jenkins Children's Hospital

Shayla Bergmann, MD, Assistant Professor Director, Pediatric Hemophilia Clinic
10 McClennan Banks Dr.
Charleston, SC 29425
Phone: 843-876-1980
Mobile: 843-812-5682
Fax: 843-792-7562

Resource Information

SC Dept. of Public Health Children with Special Health Care Needs Hemophilia Program
2100 Bull Street
Columbia, SC 29201
Phone: 803-898-0784
Website: dph.sc.gov/health-wellness/child-teen-health/services-children-and-youth-special-health-care-needs/hemophilia

American Pain Foundation (APF)
Phone: 888-615-PAIN (7246)
Hemophilia Chronic Pain Support Group
Website: painaid.painfoundation.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

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: panfoundation.org
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.

Accessia Health
Financial assistance to people who are diagnosed with chronic medical conditions.
P.O. Box 5930
Midlothian, VA 23112
Phone: 800-366-7741
Website: www.accessiahealth.org/

The Assistance Fund - TAF
8427 Southpark Circle, Suite 100
Orlando, FL 32819
Phone: 855-845-3663
Website: www.tafcares.org

Resource Information

Patient Notification System
The Patient Notification System is a free, confidential, 24-hour communication system providing information on therapy withdrawals and recalls.
Phone: 888-UPDATE U (873-2838)
Website:
www.patientnotificationsystem.org

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

The South Carolina Rare Disease Advisory Council
Website: www.rarediseasesc.org/

SC Healthcare Marketplace
Phone: 800-318-2596
Website: www.healthcare.gov

National Organizations

National Bleeding Disorders 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@Bleeding.org
Website: www.Bleeding.org

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

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

Bleeding Disorders Association of SC
Green Gate Office Park
25 Woods Lake Road, Ste. 300
Greenville, SC. 29607
Phone: 864-350-9941
Website: www.bda-sc.org

Mark Your Calendar!

Looking Ahead: 2025 Major Community Engagement Opportunities - Save the Dates!

February 25 - 26th: BDASC's State Advocacy Days, Columbia, SC
March 5 - 7th: NBDF Washington Days, Washington, DC
March 27 - 30th: HFA's Annual Symposium, San Diego, CA
April 4 - 6th: BDASC's Adult Connections Retreat, Greenville, SC
May 10th: STEP For Bleeding Disorders Walk / 5K Fundraiser, Columbia, SC
June 6 - 7th: HELLO Bleeding Disorders Conference, Greenville, SC
July 31 - August 3rd: Teen Retreat, Rockfish Camp and Retreat, NC
September 26th: BDASC's Par for the Clot Charity Golf Tournament, Greenville, SC
August 21 - 23rd: NBDF's Bleeding Disorders Conference, Denver, CO

Be sure to check out our website, e-blast newsletters and social media for registrations and updated information on upcoming events.

Additional Ways to Support BDASC

BDASC

Visit our website at bda-sc.org (or scan the QR code) and click "Make a Donation" to learn more about how you can support BDASC!

Purchase an Official BDASC Jacket and help us raise funds for financial assistance. Order online from the BDASC Apparel Store.

United Way

Donate through United Way! Go to Donate through the United Way. Visit their website at www.unitedway.org. Be sure to ask to have BDASC as your planned giving or workplace campaign.

Bleeding Disorders Association of South Carolina
Green Gate Office Park
25 Woods Lake Road, Ste. 300
Greenville, SC 29607
www.bda-sc.org

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STEP
for bleeding disorders

P -PROMOTING RESEARCH
E -EMPOWERING PEOPLE
T -TRANSFORMING TOMORROWS
S -SHARING STORIES

5K WALK/RUN FUNDRAISER
MAY 10TH, 2025
SALUDA SHOALS PARK, COLUMBIA SC

Scan the QR Code to learn more and register or visit:
http://tinyurl.com/2025STEPForBleedingDisorders