

THE CONCENTRATE

THE OFFICIAL NEWSLETTER FOR HEMOPHILIA OF NORTH CAROLINA

SUMMER 2016

Going Down in History: 43rd HNC Annual Meeting

The 2016 Hemophilia of North Carolina (HNC) Annual Meeting was an equally moving and educational coming together of members. Beginning on Saturday morning, May 14, at the Sheraton Greensboro Hotel, the meeting was set into motion with the introduction of a very special keynote speaker, Jeanne White-Ginder, mother of Ryan White. Her touching testimony about the journey she made with her son and family carried HNC's members back to a very emotional and uncertain time impacting those affected by bleeding disorders.

It was a fitting start to the event's theme, Remembering Our Past, Moving to the Future. Many members recalled the media sensation that surrounded Ryan and Jeanne through the 1980s as they navigated Ryan's hemophilia treatments and subsequent diagnosis of HIV/AIDS acquired from a contaminated blood product. Her telling of the emotional

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Meet our New Board Members

HNC would like to welcome three new members to the Board of Directors. Please take a moment to read about and get to know them. Their commitment to HNC and its members is greatly appreciated as they begin their new positions.



Rick Clark, from Knightdale, is a graduate of Campbell University holding an Associates in Arts and Bachelor of Business Administration degrees with a concentration in Accounting and Economics. He is currently employed as an AR/Credit & Collections Account Analyst position for a privately owned, yet global operated company headquartered in Cary. Rick's relationship with hemophilia began 24 years ago, when his son Andrew Dylan Clark was born. Rick says of this time, "This was a new world to us all, as there was no previous family history and often felt like we were drinking from a fire hose while raising our son."



Tyronna Hooker, MEd, from Graham, NC, is a former North Carolina Teacher of the Year and is the current Director of District and School Partnerships for Teach For America, Eastern North Carolina. Upon completing high school in Elizabethtown, NC, she went on to earn a Bachelor of Science degree in criminal justice from North Carolina Central University. After earning her teacher certification, she earned a

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Save The Date

HNC/HSC Teen Retreat

Camp Canaan, Rock Hill, SC
July 7-10, 2016

NHF Annual Meeting

Orlando, FL
July 21-23, 2016

WFH Congress

Orlando, FL
July 24-28, 2016

Family Fun Day

Tanglewood Park
Clemmons, NC
August 21, 2016

Blood Brotherhood Retreat

Beach Location
September 23-25, 2016

HNC Family Retreat

Lake Junaluska, NC
September 30-October 2,
2016

Inhibitor Family Camp

Victory Junction
October 7-9, 2016

Raleigh Walk

Morrisville, NC
October 15, 2016

HNC Yard Sale

Morrisville, NC
October 22, 2016

HNC Blood Brotherhood

Winter Warm-up
Chapel Hill, NC
November 19, 2016

HNC Holiday Celebration

Jamestown, NC
December 3, 2016



*Hemophilia of
North Carolina*

260 Town Hall Dr., Suite A
Morrisville, NC 27560
(800) 990-5557
info@hemophilia-nc.org
www.hemophilia-nc.org

MISSION STATEMENT

Hemophilia of North Carolina is a non-profit organization dedicated to improving the quality of life of persons affected by bleeding disorders through advocacy, education, promotion of research and supportive programs and services.

Contact Numbers

Hemophilia of North Carolina

(800) 990-5557
(919) 319-0014
(919) 319-0016 (fax)

National Hemophilia Foundation

(800) 42-HANDI
www.hemophilia.org

Hemophilia Federation of America

(800) 230-9797
www.hemophiliafed.org

Community Health Charities of NC

(919) 554-3272 (Collect)
www.healthcharities.org

About This Publication

THE CONCENTRATE is the official newsletter for Hemophilia of North Carolina. It is produced quarterly and distributed free of charge to requesting members of the bleeding disorder community.

An electronic version may be found on the HNC website. If you would prefer not to receive a mailed copy of our newsletter, please contact the HNC office.

We maintain a membership mailing list. However, we never release any personal information without your permission.

Hemophilia of North Carolina does not endorse any specific products or services and always recommends that you consult your physician or local treatment center before pursuing any course of treatment.

Hemophilia Treatment Centers

East Carolina University Brody School of Medicine

600 Moye Boulevard
Pediatric Hematology/Oncology
MA Suite 333
Greenville, NC 27834
Phone: (252) 744-4676
Fax: (252) 744-8199

University of North Carolina Hemophilia and Thrombosis Center

UNC Hemophilia and Thrombosis Center
170 Manning Drive
3rd Floor Physicians Office Building
Campus Box 7016
Chapel Hill, NC 27599-7016
Phone: (919) 966-4736
htcenter.med.unc.edu

Wake Forest University Baptist Medical Center

The Bowman Gray Campus
Department of Pediatrics
Medical Center Boulevard
Winston-Salem, NC 27157-1081
Phone: (336) 716-4324
Fax: (336) 716-3010

Additional Medical Resources

Carolinas Medical Center Pediatric Hematology/Oncology

1000 Blythe Boulevard
Charlotte, NC 28203
Phone: (704) 381-6800

Duke University Medical Center Hemostasis and Thrombosis Center

DUMC Box 3422
Durham, NC 27710
Phone: (919) 684-5350
htc.medicine.duke.edu

St. Jude Affiliate Clinic at Novant Health Hemby Children's Hospital

Hematology & Oncology Clinic
301 Hawthorne Lane, Suite 100
Charlotte, NC 28204
Phone: (704) 384-1900

Resource Information

National Hemophilia Foundation

www.hemophilia.org

Hemophilia Foundation of America

www.hemophiliafed.org

American Society of Pediatric Hematology/Oncology

847-275-4716
www.aspho.org

Centers for Disease Control & Prevention

1-800-311-3435
www.cdc.gov

Coalition for Hemophilia B

1-212-520-8272
www.coalitionforhemophiliab.org

ClinicalTrials.gov

A registry of federally and privately supported clinical trials conducted and service of the US National Institutes of Health. It gives you information about a trial's purpose, who may participate, locations, and phone numbers for more details. This information should be used in conjunction with advice from health professionals.

Committee of Ten Thousand (COTT)

1-800-488-2688
www.cott1.org

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201-493-1399
www.inalex.com

LA Kelley Communications

1-978-352-7657
www.kelleycom.com

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www.patientnotificationsystem.org

Patient Services Incorporated (PSI)

Assists persons with chronic medical illnesses in accessing health insurance and pharmacy co-payment assistance.
1-800-366-7741
www.uneedpsi.org

World Federation of Hemophilia

1-800-520-6154
www.wfh.org

VISION STATEMENT

Hemophilia of North Carolina's vision is for all persons affected by bleeding disorders to achieve their full potential without barriers or limitations.

Hemophilia of North Carolina is a chapter of the National Hemophilia Foundation, a chapter member of the Hemophilia Federation of America, a member agency of Community Health Charities, a member of the NC Center for Nonprofits.



Meet our New Board Members

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Master of Education from Elon University with a specialization in special education. Tyronna is an associate pastor at Parrish Chapel UCC where she is an active member of the choir and the youth department director. She is dedicated to helping, healing and revealing the purpose God has for the youth and families. She believes that every child has the right to learn to read or someone will read them their rights. She is an innovator with bold ideas, high energy and a heart for helping children achieve their purpose. She has experienced the challenges of chronic illness firsthand because both of her sons, Myles and Kendall, have severe hemophilia.



Arlette Whitaker, RN, from Greenville, is a pediatric and adult hemophilia nurse at the East Carolina University Hemophilia Treatment Center. She has served as a clinical nurse specialist for almost three years in hemophilia and bleeding disorders. She also has been actively involved in hemophilia education and

volunteer opportunities. She has a desire to promote hemophilia awareness and education to patients, families and the community.

In addition, while we welcome our new board members, it is time to say good-bye to one who has completed his term. Dylan Clark, of Knightdale, has been a member of the HNC Board of Directors for two years as well as an active volunteer who is always willing to lend a hand when needed. Dylan was diagnosed with severe hemophilia A shortly after birth. He had a multitude of complications from having fluid on the brain, failures of several central lines, and an inhibitor. He is grateful for the love and care from doctors at the Hemophilia Treatment Center at the University of North Carolina and the continued outpouring of care and support from his extended family in the hemophilia community. Dylan's desire is to help Hemophilia of North Carolina continue to grow by pouring out that same care and support he received to the younger generation. We thank Dylan for his time and dedication on the HNC Board over the past two years.

HNC and the “Hope for Hemophilia” organization

Letter from our Board President, April 29, 2016

There has been some confusion lately regarding the activities of a new organization, Hope for Hemophilia, that has been sponsoring some social events in North Carolina. Because of the similarity of their name with HNC's HOPE program, several of our members have mistakenly assumed that their activities are sponsored or co-sponsored by HNC. HNC has not engaged in any activities with Hope for Hemophilia, and we will not be involved in any programs with them for the foreseeable future.

HOPE is an HNC program that supports families who have children with a bleeding disorder. Its purpose is to help families learn to manage their child's bleeding disorder so that it does not define his or her life. The program includes educational dinners, regional groups, and formal gatherings at major HNC events (such as the annual meeting, family retreat, and holiday dinner).

Industry representatives at all HNC-sponsored or co-sponsored events, including the HOPE program, are required to use an HNC-designed form for obtaining HNC member contact information. This is so the community member will understand that they are giving the representative permission to contact them. Company representatives are clearly identified as such at every HNC event, and must sign our “Industry Standards” document at every event. HNC does not share our members' names or contact information with industry representatives or other non-profit groups.

Hope for Hemophilia is a non-profit corporation based in Baton Rouge, Louisiana. They are primarily involved in administering a financial assistance program. They are not involved in sponsoring educational or social programs of the type HNC develops and delivers.

Every communication that you receive from HNC will have the HNC logo on it. If you are unsure about the sponsorship of any program for which you have been invited or solicited, please contact HNC with questions by emailing us at info@hemophilia-nc.org or by calling 1-800-990-5557.

It is our mission to provide the community with a safe, comfortable, and supportive environment at all of our events, and to deliver the finest educational programs to the North Carolina bleeding disorders community. Our mission is YOU!

Steve Peretti

President

Hemophilia of North Carolina

World Hemophilia Day Lights Up Charlotte

HNC proudly partnered with Biogen to support the World Federation of Hemophilia (WFH) and the global hemophilia community in recognizing World Hemophilia Day on April 17, which raises awareness for and supports individuals living with inherited bleeding disorders.

Gathering at the Fairfield Inn & Suites Charlotte Uptown, members of the community enjoyed a wonderful dinner and presentation followed by an exciting, interactive activity called the Factor Factory Challenge. Building the working machine brought the clotting process to life and demonstrated that if just one part of the progression is missing the process cannot be completed. People of all ages had fun while also learning some of the science behind the clotting process.

With the sun setting, it was time to head outside for the “highlight” of the evening and watch as the Duke Energy Building lit up the Charlotte skyline red to honor those affected by bleeding disorders worldwide. With clear skies, it was a perfect evening to enjoy each other’s company and look out over the city to see a shining symbol of the world hemophilia community coming together to support and advocate for one another.



State of North Carolina

PAT McCrORY
GOVERNOR
WORLD HEMOPHILIA DAY
2016

BY THE GOVERNOR OF THE STATE OF NORTH CAROLINA
A PROCLAMATION

WHEREAS, hemophilia is a rare genetic bleeding disorder in which one of the proteins that causes the blood to clot is missing, or there is not enough of it to work properly, causing people to bleed longer; and

WHEREAS, without effective blood clotting, cuts and internal injuries have difficulty healing, and internal bleeding into the joints can result in pain and permanent damage; and

WHEREAS, hemophilia is typically a lifelong condition which is primarily treated with infusions of a protein involved in blood clotting; and

WHEREAS, it is estimated that more than 400,000 people worldwide are living with hemophilia; and

WHEREAS, approximately 20,000 people in the United States are living with hemophilia; and

WHEREAS, approximately 1,000 people in North Carolina are living with hemophilia; and

WHEREAS, hemophilia is found in all populations; and

WHEREAS, people living with hemophilia, and those caring for and serving them, are strongly united in advocating for access to treatment and care;

NOW, THEREFORE, I, PAT McCRORY, Governor of the State of North Carolina, do hereby proclaim April 17, 2016, as “WORLD HEMOPHILIA DAY” in North Carolina, and commend its observance to all citizens.

IN WITNESS WHEREOF, I have hereunto set my hand and affixed the Great Seal of the State of North Carolina at the Capitol in Raleigh this fifth day of April in the year of our Lord two thousand and sixteen, and of the Independence of the United States of America the two hundred and fortieth.



Pat McCrory
PAT McCRORY
Governor

City of Charlotte, North Carolina Proclamation

WHEREAS, Hemophilia is a rare genetic bleeding disorder in which one of the proteins that causes the blood to clot is missing, or there is not enough of it to work properly, causing people to bleed longer^{1,2,3}; and

WHEREAS, Without effective blood clotting, cuts and internal injuries have difficulty healing, and internal bleeding into the joints can result in pain and permanent damage^{1,2,3,4,5}; and

WHEREAS, Hemophilia is typically a lifelong condition¹, which is primarily treated with infusions of a protein involved in blood clotting^{1,2}; and

WHEREAS, It is estimated that more than 400,000 people worldwide are living with hemophilia¹; and

WHEREAS, Approximately 20,000 people in the United States are living with hemophilia¹; and

WHEREAS, Approximately 1,000 people in North Carolina are living with hemophilia⁶; and

WHEREAS, Hemophilia is found in all populations²; and

WHEREAS, People living with hemophilia, and those caring for and serving them, are strongly united in advocating for access to treatment and care;

NOW, THEREFORE, I, Jennifer Watson Roberts, Mayor of Charlotte, do hereby proclaim April 17, 2016 as

“WORLD HEMOPHILIA DAY”

in Charlotte and commend its observance to all citizens.

WITNESS MY HAND and the official Seal of the City of Charlotte.



Jennifer W. Roberts
Jennifer Watson Roberts
Mayor

2016 Volunteer and Special Guest Dinner



As traditionally held the evening before the Annual Meeting, this year's celebration of HNC's volunteers and special guests took place on Friday, May 13th at the Sheraton Greensboro Hotel. Guests were celebrated for their time and dedication to HNC's overall mission of improving the quality of life of persons affected by bleeding disorders.

The dinner featured a touching presentation from Jeanne White-Ginder, mother of Ryan White. She told the story of how her life went from just being a mom of a child with hemophilia to

being the mother of a famous child who personified the fears of a misunderstood disease, HIV/AIDS. Through their fight for Ryan's right to attend school, they also brought attention to the need to keep the blood supply safe for all recipients of blood products, especially the hemophilia community.

It was fitting to highlight Jeanne White-Ginder's contributions to the bleeding disorders community at an event held to recognize those who most contributed to HNC's success this past year. HNC appreciates the overwhelming support it receives through the efforts of so many individuals, from members to our community partners and supporters. This dinner is one small way to show them how grateful HNC is for their continued support.

There are many ways people around the state can become a volunteer or contribute their talents to help HNC promote advocacy, education, promotion of research, and delivery of supportive programs and services throughout the state. If you would like to find out more about these opportunities, please call the HNC office (800) 990-5557 or email info@hemophilia-nc.org for additional information.



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HNC Travels Around the State in June

June wraps up another busy quarter here at Hemophilia of North Carolina (HNC) with several programming events!

The Men's Educational Retreat took place on June 10-12 at Blowing Rock Conference Center, Blowing Rock, NC. This is the second annual educational event to support the men in the community, both fathers of children affected by and men who themselves are affected by bleeding disorders. Programming designed to meet the needs of each group was available as well as activities that benefit all.

Our State Legislative Day was on June 14 in Raleigh. Starting with a training dinner the evening before, the agenda included an educational program to help participants learn more about advocacy. These programs, *Do's and Don'ts & Telling Your Story*, are designed to help members gain confidence and understanding about how to talk to legislators. Then, on June 14th, members visited their NC representatives to discuss what is important to them as well as members of the entire community of those affected by bleeding disorders in North Carolina.

HNC's Latin Union hosted a dinner and ballgame on Saturday, June 18 in Charlotte. This program included dinner and a Baxalta Hello Talks! presentation, *La hemofilia y un sano envejecimiento (Healthy Aging and Hemophilia)*, followed by a trip across the street to see the Charlotte Knights take on the Indianapolis Indians.

On Saturday, June 25th, members of HNC gathered for fun and BBQ in Morrisville, NC. HNC member and king of the grill, Jeff Neal, is hauled his famous cooker to the HNC office to help celebrate the wonderful community we belong to. With barbecue pork and chicken, corn on the cob, coleslaw, beans and more, nobody would go hungry. There was also corn hole and other activities for the kids.

The next issue of *The Concentrate* will have updates and recaps for you on these programs and more!

Going Down in History: 43rd HNC Annual Meeting *continued from page 1*



road to becoming an advocate touched every heart in the room and reminded members of the many obstacles those like the White family and others living with bleeding disorders had to overcome in the past to bring the community to where it is today.

Following the Keynote Address, attendees were fortunate to have Kristy Lee, MS CGC, Associate Professor in the Department of Genetics at UNC Chapel Hill, discuss the genetics behind bleeding disorders so members could all better understand the science behind their diagnosis. Genetics can be a daunting topic but Kristy does an exceptional job of presenting the information in a way that everyone can understand. Of course there are always questions, which she was happy to answer!

New this year was an awards luncheon to honor those who have participated in HNC's Legislative Day and/or NHF's Washington Days in the last twelve months. Many of these individuals and families have been advocating on behalf of the community for many years and HNC was proud to be able to recognize them on such a memorable occasion. There was a special award for Jeanne, for all that she and her family did to pave the way for generations to come.

After a delicious lunch, the HNC Business Meeting was held. Various topics were discussed pertaining to HNC's past year and an election was held for new board members. Following the business meeting, members were able to hear an inspiring presentation by Sonji Wilkes of Hemophilia Federation of America called *Honoring our Past, Building our Future*, which corresponded nicely with the banner display of, "Historical Journey of Bleeding Disorders" that was viewable throughout the entire day.

Breakout sessions were held for different HNC Programs. Morning sessions included: SOAR (women & girls), Blood Brotherhood (adult men), Spouses/Partners, and HOPE (for families of young children). Afternoon breakout sessions were held for other areas

of interest including: Social Media, the Latin Union Program (Spanish-speaking individuals), von Willebrand's Disease and Inhibitors. Thank you to our speakers: Charlene Cowell, Patricia Espinosa, Crystal Hoernlein, Alex Ibarra, Matt Igelman, Sharon Ingram, MA HS-BCP CWHC, Jim Munn, RN, Gillian Schultz, and Sonji Wilkes.

Children and teens were also kept busy with plenty of fun-filled activities targeted toward the different age groups present. Younger kids were able to participate in programs like *Mad Science; My Amazing Blood, Blood Sundaes; Learning About YOU; Cold Blooded Encounters*, along with other games and crafts. Teens' programs included *HFA History; Self Advocacy; Junior Scientist* and participation in some of the adult sessions to learn about the history of bleeding disorders. A big thank-you goes out to all the presenters of our kids' and teens' programs: Elizabeth Patrick, Cathy Harber RN, Ana Garcia, RN BSN, Sonji Wilkes, Patricia Espinosa and Crystal Blankenship PharmD, for providing these fun learning opportunities to HNC's younger members. Childcare was also offered for infant children so adults could benefit from the programming worry-free. Thank you to everyone who made the infant, children and teen programs possible!

Hemophilia of North Carolina gratefully acknowledges the pledge of support from the following companies for the 2016 HNC Annual Meeting:

Platinum & Volunteer Dinner Sponsors



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- Kedron Biopharma • Matrix Health • Octapharma
- Option Care • Pfizer Hemophilia

The 2nd Charlotte Walk Hits the Streets



It was a glorious morning for a walk in Uptown Charlotte for Hemophilia of North Carolina's 2nd Annual Charlotte Walk. With more than 500 people checked in and over \$48,000 raised, the bleeding disorders community has truly made our mark in the Charlotte area which could not have been done without each and every person who contributed in some way. Thank you to all of the walkers, volunteers, team captains, sponsors, supporters and members of the community who came out to make this event a great experience for everyone.

The streets of Uptown Charlotte were quiet and still when the volunteers arrived before sunrise to set up for the day. But the calm did not last as it wasn't too long after daybreak that the street was completely transformed into a block party. With activities and booths lining the street, walkers and friends could enjoy crafts, face-painting, the photo booth, sno-cones, try out their hockey skills or even throw a pie in the face of one of HNC's staff or board members! Local Charlotte celebrities were on hand as well with special appearances by the event emcee, Larry Sprinkle, Morning Forecaster for NBC Charlotte and Charlotte Knights' Mascot Homer the Dragon. Even with all that fun at hand, the Walk would not have been the same without HNC's long-time supporters, Jecoriography and DJ Kevin McVerry turning up the excitement.

It could not be ignored that the Walk was hosted on the day before World Hemophilia Day. As such, the Walk was themed around this important event and included turning the usual HNC booth into a Journey Around the World display. With information and giveaways from all over, everyone had an opportunity to learn more about bleeding disorders worldwide. There were also craft boxes, foldable

2nd Charlotte Walk Hits the Streets Continued on page 9

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Latin Union Group is Hanging with the Hippos



Members of the Latin Union Program went for a walk in the wild during the April 9th event at the NC Zoo in Asheboro. Starting at the Hippo Beach Picnic Shelter, all of the families gathered and the kids had some downtime to play hide-and-go-seek. They climbed on the giant hippo for some photos, which worked up their appetites. After a delicious picnic lunch it was time to start the program, which included a Baxalta Hello Talks! presentation, *No Sweat: Staying Active and Healthy with a Bleeding Condition*. Once everyone knew the importance of exercising, it was time to hit the zoo for some physical activity and animal-spotting! HNC would like to thank Latin Union Members and Baxalta for supporting this event.

Teen Retreat: Come Join in the Fun!

Camp Canaan – Rock Hill, SC
July 7-10, 2016

Hemophilia of North Carolina is once again partnering with Hemophilia of South Carolina for this year's Teen Retreat at Camp Canaan in Rock Hill, SC, from July 7-10, 2016.

Who can attend? Anyone age 13-18 who has either a bleeding disorder, or a sister, brother or parent with a bleeding disorder can participate in this fun-filled weekend of activities.

HNC and HSC are planning a great weekend for teens to spend with friends, new and old. Activities are designed to challenge, inform and inspire. It's a time to share interests, passions and ideas about how to make a difference in the community with programs led by Joe Torrey, Pat "Big Dog" Torrey's younger brother. Can't wait to hear what his nickname is!

The weekend is free of charge and includes lodging, bus transportation, meals and activities; so bring a friend and join the fun at this year's Teen Retreat!



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0215-00025301-2 April 2015



The 2nd Charlotte Walk Hits the Streets

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fans, and other items that were representative of the fact that the next day, April 17th, was World Hemophilia Day. A great thanks to Richard Atwood, past HNC president and longtime volunteer, and Adriana Henderson, president and founder of Camp Ray of Hope in Romania, for managing the booth and all of the foot traffic!

This location proved to be the right place to build awareness for Bleeding Disorders right in the middle of North Carolina's largest city. With the adjacent park and both residential and commercial buildings surrounding the Walk site and route, people in the area couldn't help but be drawn in to the party and ask questions about the event – just one more measure of the day's success! Speaking of success, there were some HNC members and supporters who received special recognition for their efforts in making the 2nd Annual Charlotte

Walk a day to remember! Congratulations to Team Gman, top team fundraiser, and Erin Otey, top individual fundraiser. Also, a special thanks to the extraordinary efforts of our 2016 Droplet Award Winners: Kings Andrew and Jeron Hill, Queen Andrea Mayle and Junior Droplet Grayson Odom. Finally, HNC would like to recognize our first annual Charlotte Walk Hospital Cup winner, St. Jude Affiliate Clinic at Novant Health Hemby Children's Hospital. HNC would also like to thank Jordana Zeger, NHF Chief Operating Officer for her support and joining us in Charlotte this year.



Want to Know More

about your factor options?



When it comes to your factor therapy, you have choices. And at Biogen, we recognize the importance of continued research as well as supporting the hemophilia community. See if now might be the right time for you to make a change—learn more about our therapy options as well as our range of financial, educational, and community support programs.

To learn more about these options, contact your CoRe Manager:

Tanya Stephenson | **Phone:** 704.293.6925 | **E-mail:** Tanya.Stephenson@biogen.com

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A través de la Historia: 43 reuniones anual de HNC

La reunión anual del 2016 de la asociación de Hemofilia del Norte de Carolina (HNC) fue igualmente conmovedora y educativa para los miembros. En la mañana del sábado, 14 de Mayo, en el hotel Sheraton Greensboro Hotel, la reunión empezó con la introducción de nuestra especial invitada, Jeanne White-Ginder, madre de Ryan White. Su conmovedor testimonio sobre el recorrido que hizo con su hijo y familia llevó a los miembros de HNC a regresar a un momento muy emocional e incierto que afectó a las personas con trastornos de la coagulación.

Su testimonio encajo perfectamente con el tema de la reunión: *Recordar nuestro pasado, Movernos hacia el futuro*. Muchos miembros recordaron la cobertura de los medios de comunicación sobre la historia de Ryan y Jeanne a través de la década de 1980 y como Ryan luchó buscando tratamientos para su hemofilia y posteriormente su VIH/SIDA adquirida a través de un producto que tenía sangre contaminada. Su relato sobre el recorrido que tuvo que hacer para convertirse en una defensora, tocó todos los corazones en la sala y recordó a los miembros de HNC de los muchos obstáculos que tuvieron que superar en el pasado la familia White y muchos otros

personas con trastornos sanguíneos para llevar la comunidad a donde está hoy.

Después de la charla, tuvimos la suerte de que Kristy Lee, MS CGC, profesor asociado del Departamento de genética de UNC Chapel Hill viniera a discutir la genética detrás de los trastornos de la coagulación para que los miembros pudieran entender la ciencia detrás de su diagnóstico. Genética puede ser un tema difícil pero Kristy hace un trabajo excepcional de presentar la información de una manera que todos pueden entender. Por supuesto, siempre hay preguntas, que ella estaba feliz de responder!

En este año durante el almuerzo se hizo una premiación para honrar a los miembros que han participado en los últimos doce meses en el día legislativo de HNC o en los días de Washington patrocinados por NHF. Muchas de estas personas y sus familias han estado abogando en nombre de la comunidad durante muchos años y HNC se enorgullece de reconocerlos en una ocasión tan memorable. Hubo un premio especial para Jeanne, por todo lo que ella y su familia han hecho para futuras generaciones.

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A través de la Historia: 43 reuniones anual de HNC continued from page 10

Después de un delicioso almuerzo, se llevó a cabo la reunión de negocios de HNC donde se discutieron diversos temas relacionados con actividades del pasado año y se eligieron nuevos miembros de la junta directiva. Después de la reunión de negocios, los miembros pudieron escuchar la presentación de Sonji Wilkes de la Federación de hemofilia de América: *Honrando nuestro pasado, construyendo nuestro futuro*, que correspondió muy bien con la exhibición que tuvimos durante todo el día llamada: "Recorrido Histórico de los desórdenes de sangrado".

Sesiones educativas se llevaron a cabo para diferentes programas de HNC. Sesiones de la mañana incluían: SOAR (mujeres y niñas), Hermandad de sangre (hombres adultos), cónyuges/parejas, y HOPE (para familias de niños pequeños). Después de la pausa del almuerzo, se celebraron sesiones de otras áreas de interés incluyendo: el programa de Unión Latina (personas de habla hispana), la enfermedad de von Willebrand's e inhibidores. Gracias a nuestros ponentes: Charlene Cowell, Crystal Hoernlein, Matt Igelman, Sharon Ingram, MA HS-BCP CWHC, Gillian Schultz, Patricia Espinosa, Alex Ibarra, Sonji Wilkes y Jim Munn, RN.

Niños y adolescentes también se mantuvieron ocupados con una variedad de divertidas actividades dirigidas hacia los diferentes grupos de edades. Los niños más pequeños participaron en programas como Mad Science; *My Amazing Blood*, *Blood Sundaes*; *Learning about you*; *Cold Blood Encounters*, junto con otros juegos y manualidades.

Los programas para adolescentes incluían: HFA History, Self Advocacy, Junior Scientist y también participaron en algunas de las sesiones de adultos para aprender sobre la historia de trastornos de la coagulación. Muchas "gracias" va para todos los presentadores de los programas de niños y adolescentes: Elizabeth Patrick, Cathy Harber RN, Ana Garcia, RN BSN, Sonji Wilkes, Patricia Espinosa y Crystal Blankenship PharmD, por hacer posible estas divertidas oportunidades de aprendizaje a nuestros miembros jóvenes. También ofrecimos servicios para niño infantiles para que sus padres pudieran participar en los diversos programas libre de preocupaciones. Gracias a todos los que hicieron posible los programas de bebés, niños y adolescentes

Grupo de la Unión Latina se reunió con los hipopótamos

Los miembros de la Unión Latina pudieron caminar en la selva dentro del zoológico del Norte de Carolina ubicado en Asheboro. El grupo se reunió en el área de "Hippo Beach Picnic". Mientras las familias se reunían los niños tuvieron tiempo de jugar a las escondidas y subirse al hipopótamo gigante para tomar fotos. Estas actividades les abrió el apetito. Después de un delicioso picnic fue tiempo de iniciar el programa, que incluyó una presentación de Baxalta: *No sude: manténgase activo y saludable con un trastorno sanguíneo*. Una vez que todo el mundo entendió sobre la importancia de hacer ejercicio, era ya hora de ir al zoo para empezar una actividad física y observar los animales!



Cena educativa de la Unión Latina

La Unión Latina tiene otro evento divertido el 18 de junio en Charlotte. Cinco familias se inscribieron para una cena de deliciosa pasta y de un programa educativo de Baxalta sobre: *envejecer en forma saludable y la hemofilia*. Patricia Espinosa-Thomson planea discutir cómo permanecer saludable en las diferentes etapas de la vida.

Después de la cena, el grupo está programado para ver el juego de los Charlotte Knights contra los Indios de Indianapolis. Ganar o perder siempre es agradable salir afuera y ver un partido de béisbol en el BB & T Ballpark en el corazón de uptown Charlotte.

Nos gustaría agradecer a Baxalta por su continuo apoyo para los programas de la Unión Latina de HNC.

Success Stories from the 2016 NP Symposium

It was a productive weekend at the beach for Hemophilia of North Carolina (HNC) as more than 300 Nurse Practitioners gathered in Wilmington for the 2016 Nurse Practitioner Spring Symposium (NPSS) on April 3-6. HNC is a proud supporter of the nursing conferences that take place throughout the state and for various specialties within the healthcare field. This offers an opportunity to exhibit and educate those on the front lines to ensure proper diagnosis and care for those living with bleeding disorders.

The NPSS was no different and HNC was glad to see so many new and familiar faces. Within the first day, over 200 nurses had stopped by to pick up some materials about bleeding disorders to read and bring back to their colleagues and friends. Nurses came by to thank HNC for continued support of these conferences because it helped in the diagnosis of multiple people throughout NC, including one of the nurse practitioners in attendance! HNC looks forward to the next nurse's conference and hopefully hearing more success stories. HNC also wants to acknowledge the volunteers who helped prepare for and exhibit at these events and also CSL Behring, who has provided funding that allows us to continue in these important efforts!



Membership Information

For more information about SOAR, or to be added to the SOAR mailing list, please send your name and contact information, along with any questions you may have, to soar@hemophilia-nc.org.

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An HNC Program for Girls and Women with Bleeding Disorders

SOAR's Mission Statement: *To improve the quality of life for girls and women with bleeding disorders, so that they may SOAR to their full potential.*

SOAR Members at the NOW Conference

By: Cheri Clark



I, along with SOAR members Crystal Hoernlein and Heidi Traficanti, had the pleasure of attending the 4th Annual NOW Conference at the Pointe Hilton Squaw Peak in sunny Phoenix, AZ April 22-24th. NOW stands for National Outreach for von Willebrand and is an educational conference hosted by the Arizona Hemophilia Association

(AHA) focused specifically on von Willebrand disease, the most common hereditary bleeding disorder. This forum unites over 200 men, women and children with vWD from all over the country in order to network, learn of the newest treatment options, and gain the tools necessary to better manage our disease.

Medical experts including hematologists from several of the hemophilia treatment centers, nurse practitioners, therapists

and many others shared their knowledge with us through large group sessions that included the basics of vWD and numerous breakout sessions tailored to our particular interests. There was also a physician panel discussion where experts in the field answered questions from the conference participants.

As a female with vWD and a member of SOAR, I found NOW to be a wealth of valuable information. I was not only able to gather more information related to my own vWD, but increased my knowledge base in general in order to help spread awareness on vWD through the volunteer work I do with NC SOAR.

I would strongly recommend that anyone with vWD try to attend this extremely well-organized and informative conference. Upcoming conferences will be in November 2016 and in April 2017. Registration information can be found on the AHA site.

A special thank you goes out to the Arizona Hemophilia Association for hosting this fantastic event and to CSL Behring for funding. I came back to NC more knowledgeable, more relaxed and a bit sunburned!

NOW Conference: A Spouse's View

By: Mike Hoernlein

In April, my family and I attended the National Outreach von Willebrand (NOW) Conference in Phoenix, Arizona. NOW is an educational conference, organized by the Arizona Hemophilia Association and generously funded by CSL Behring. This conference focuses exclusively on Von Willebrand Disease.

The weekend was packed with presentations and panel discussions addressing a wide range of topics, from coping with the various challenges of living with bleeding disorders to recent medical advances. Because the schedule included several blocks of small sessions on different topics, that were offered simultaneously and then repeated once, attendees could really tailor their experience to suit their own needs and interests.

The speakers were uniformly impressive. In addition to deepening my understanding of a chronic disease that my wife and son live with and getting practical advice from experts in the field, I got to meet and spend time with families from around the country who have their own stories to share. The conference really exceeded all of my expectations.

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Interview with Riley and Noah Schultz



Riley (8) and Noah (5) love swimming. While a fun summer activity for most kids, it is also an excellent sport to participate in when you have a bleeding disorder. The boys have been going to their neighborhood pool since they were babies and this summer have joined their neighborhood swim team, The Barracudas.

When did you first learn to swim?

Riley: When I had swimming lessons for the last 2 summers.
Noah: I learned to swim last summer.

Why are you on swim team?

Riley: Because my mom put me on the team. But I didn't want to be on swim team.
Noah: Because I wanted to. I thought it would be fun.

What do you do at swim practice?

Riley: We practice starts, finishes, and diving. We jump in feet first in the shallow end and start swimming and dive in the deep end. We swim laps, the warm up is 6-8 25s (one length of the pool).

Noah: I learn how to stay under water, jump in the water, swim with the kickboards, learn how to move my arms to swim without the kickboards.

What is your favorite part of swim team?

Riley: My favorite part of swim team is butterfly.
Noah: My favorite part is jumping in from the deep end.

Why is that your favorite part of swim team?

Riley: Because it's challenge level is in the middle.
Noah: Because you can do more stuff in the deep end.

What is the most challenging part of swim team?

Riley: The most challenging part is backstroke.
Noah: Swimming an entire lap, starting from one side and going all the way to the other side.

Why is that the most challenging part of swim team?

Riley: Because you have to do three things at a time, make

your arms go in backwards circles, float on your back, and kick underwater.

Noah: Because I have to take a breath a lot of times, maybe 6 times and it's tiring.

What do you think you will do at the swim meets?

Riley: Races.
Noah: Swim laps.

Are you excited for swim meets?

Riley: Truthfully, I don't know.
Noah: Yes.

Will you continue to swim the rest of the summer, after swim team is over?

Riley: Yes.
Noah: I guess.

Why do you think it is healthy to swim?

Riley: Because it makes you stronger.
Noah: Because I have hemophilia and it makes me strong.

Do you think you will join the swim team next year?

Riley: No, because I don't like being coached to swim, I like free swimming more because I can do what I want.
Noah: I'm not sure yet.

Never Too Young To Volunteer!

By: Sienna Hoernlein

My name is Sienna Hoernlein. I volunteer for HNC because my mom and brother have von Willebrand Disease, and because IT'S FUN! I've helped fold folders for a nurse's conference, make favors for casino night, and make tutus for the walks. It's really fun at the walks, because there is usually a bouncy house, a snow cone stand, and all that fun stuff. I helped with the chocolates for casino night, because I really wanted to eat them. In the future I'd like to help out with one of the walks. I like to help do things, because it's fun!



HNC Volunteers come in all ages! Thanks to Sienna Hoernlein for her hard work getting materials ready for the HNC Booth at the Nurse's. If you want to find out more about HNC Volunteer opportunities, please call the HNC office.

A Sad Goodbye to Two Members of Our Community

It is with deep sadness that we announce the passing of two members of the Hemophilia of North Carolina community.



Andrew (AJ) Hill, 21, passed away on Friday, May 19th at the CaroMont Regional Medical Center in Gastonia. AJ and his family have been valued members of the HNC community for many years and have been instrumental in making the Charlotte Walks a success through their walk team, Team Hill. This earned AJ and his brother, Jeron, the King Droplet award at the 2016 Charlotte Walk. AJ's smile and vivacious personality would light up the room. He will be sorely missed by all.



Christine Anna Johnson, MD, Emeritus Professor of Pediatrics at Wake Forest University School of Medicine, age 77, died peacefully on Sunday May 8th following a protracted illness. Through her dedication, perseverance, and hard work, Dr. Johnson was awarded federal and state grant funding,

which allowed her to establish the Comprehensive Hemophilia Diagnostic and Treatment Center and the Comprehensive Pediatric Sickle Cell Disease Program at Wake Forest University School of Medicine. She served as medical director for these programs until her retirement in 2003.

Dr. Johnson was a role model for those with whom she worked. She was a brilliant and creative academic physician, who excelled in teaching and in patient care. She was loved and respected by her patients, colleagues, and house staff. She had a tireless work ethic and a great sense of humor. Despite all her gifts, she remained humble. She set an example for what it means to be a complete physician.

You can read her very interesting obituary in its entirety on the Salem Funeral Home website, www.salemfh.com/obituaries/3070. We are grateful to Dr. Johnson for her tireless efforts to improve the quality of lives for those affected by bleeding disorders.

Family Retreat

Lake Junaluska Conference and Retreat Center—NC Mountains
September 30 – October 2, 2016

Mark your calendars for September 30 – October 2 for HNC's annual Family Retreat. This year's retreat will be returning to Lake Junaluska Conference and Retreat Center in the NC Mountains. Families who have a child(ren) through the age of 12 who is affected by a bleeding disorder should join us for this event as programming will be geared towards this age range! The weekend will be filled with fun and educational activities. There will be sessions for adults, and activities for kids, both affected and unaffected, as well as childcare for all ages. More information and registration will be available soon on the HNC website, so save the date!

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Walk for a Cure in Raleigh

HNC Raleigh Walk, October 15, 2016

Lake Crabtree County Park, Morrisville, NC

It's hard to imagine a brisk autumn day while we're still trying to stay cool this summer, but it's not too soon to start getting ready for the 9th Annual Hemophilia Walk in Morrisville this October! This is the largest fundraising event for HNC each year, and it would be great to see you there!

This year's walk will take place on Saturday, October 15th at Lake Crabtree County Park in Morrisville. Have you set up your walk team? Information is available in English and Spanish at www.hemophilia-nc.org to help you kick start your team! The site features information for walkers, sponsors, teams and volunteers.

The day will feature a 5K walk, food, music, games and all-around fun for family and friends (even the 4-legged ones). Tell your friends, families, neighbors and coworkers so they can save the date, and join in the fun!

Printed Sponsorship and Team Captain Packets are also available through HNC by calling 800-990-5557 or by email at info@hemophilia-nc.org. We are looking forward to another great event this year and hope to see everyone there!

HNC Holiday Celebration

December 3, 2016

Jamestown, NC

Mark your calendars! HNC will once again host its Holiday Celebration at the Castle McCulloch on December 3 in Jamestown (near Winston-Salem). It is a beautiful setting to start off the holiday season with your HNC family and a visit from our special friend from the North Pole – also known as Santa! There will be presents for all the kids and even some prizes for the adults. Stay tuned to the HNC website for more details and registration information. Can't wait to spend the holidays with you and yours!

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Blood Brotherhood Update

The Blood Brotherhood program continues to grow here in NC!

Blood Brothers and their guests gathered for a movie night in Uptown Charlotte on April 16th. The guys got to relax and enjoy a movie and dinner after attending HNC's Charlotte Walk event. Then, in May, everyone got together at HNC's Annual Meeting where they were able to share their experiences with one another during the morning Blood Brotherhood breakout session. Most recently, at the Men's Retreat in June, they got together again for an educational program and some fun tubing in the NC mountains. Be on the lookout for announcements about future Blood Brotherhood gatherings and events. All adult men affected by a bleeding disorder are welcome to participate.

As always, thanks to Hemophilia Federation of America (HFA) for presenting this program and opportunity here in NC and many other states. In addition to local Blood Brotherhood events, HFA offers an online forum and chat session just for Blood Brothers.

For more information on Blood Brotherhood nationally, including information on the private online forum for adult men with bleeding disorders, and the online Blood Brotherhood Chats, visit: www.hemophiliafed.org/programs/blood-brotherhood

For more information on Blood Brotherhood locally, visit the HNC website www.hemophilia-nc.org, and save these dates for 2016 Blood Brotherhood events here in NC:

September 23-25 - Beach Location TBD

November 19 - Chapel Hill, NC

And more...



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HOPE Update

- *Help*
- *Opportunity*
- *Partnership*
- *Empowerment*

Summer is here and HNC's HOPE Program is certainly heating up! There are many exciting things in store and for families to come out and participate.

For years, people in the community have been asking for a peer support and mentoring program. HNC has been working on making this a reality and will now be able to provide this program to members! HNC recently applied for and received a grant from the Hemophilia Alliance. This grant will allow us to provide thorough training for those interested in becoming a mentor, as well as to provide a structure and resources for future mentoring partnerships. Needs assessments are being sent to members of the community electronically and by mail. Please take a few minutes to complete this survey and return it as it will help to guide both planning and implementation of the mentoring program. If you have not received the needs assessment and are interested in completing it, please contact us at the office to be make sure that you have the opportunity to complete this important survey. You may also be receiving a phone call from Gillian Schultz to conduct a phone interview.

You can read about two other exciting HOPE events coming up in the next few months in this edition of the Concentrate. The first

event is a new initiative for HNC. Join us on Sunday, August 21, for our first annual Family Fun Day Out at Tanglewood Park in Clemmons, NC. The next event will be a return to Lake Junaluska Conference and Retreat Center from September 30 – October 2 for the annual Family Retreat. Stay tuned to the HNC website and Facebook pages for more information about both events.

HOPE stands for Help, Opportunity, Partnership, Empowerment. The mission of HOPE is to improve the quality of life for families of the newly diagnosed through the age of twelve with bleeding disorders, so that they may HOPE to lead a fulfilling life. Together, with a community of other parents, we can support each other so that both parent and child can feel empowered, and not limited by a bleeding disorder. Open to any member of HNC, HOPE events are geared specifically towards parents with children in this age range. For more information about the HOPE Program, or if you have ideas for programs and events, please contact Gillian Schultz, HNC Program Manager at gillian.schultz@hemophilia-nc.org or contact the HNC office at (800) 990-5557. You may also check the HNC webpage or Facebook page for updates on upcoming programs and events.

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The SIPPET Bombshell

By: Paul Clement

A bombshell was dropped at the Plenary Scientific Session of the 57th annual meeting of the American Society of Hematology (ASH) on December 6, 2015, in Orlando. Study coordinators of the SIPPET project (Study on Inhibitors in Plasma-Product Exposed Toddlers)¹ presented surprising preliminary findings: recombinant factor VIII products are associated with an 87% increased risk of inhibitor development compared to plasma-derived factor VIII products.²

In other words, for every 10 people treated with recombinant factor VIII as opposed to plasma-derived factor VIII, 1 patient can be expected to develop high-titer inhibitors.

As a parent of a toddler who does not have inhibitors, you may feel stunned, angry, or scared when you read these findings. Should you be? Before you rush to make a product change, learn how the study was conducted, what its potential shortfalls are, and why you should take a deep breath!

SHOCK AND AWE

Understandably, many consumers are concerned. Some news releases describing the study results only heightened the alarm. Hemophilia Federation of America (HFA) issued a press release requesting that National Hemophilia Foundation's (NHF) Medical and Scientific Advisory Council (MASAC) "consider the temporary suspension of recommendations... that state any preference for recombinant factor products until the results of the full SIPPET study can be reviewed."³

Is this a reasonable reaction, or is this jumping the gun? It helps to examine how the study was conducted—and why.

FIGHTING INVADERS

Why was the study looking to see if plasma-derived products are less immunogenic than recombinant products—that is, less likely to lead to developing inhibitors?

In the blood, factor VIII is normally tightly bound to another protein called von Willebrand factor (VWF). VWF has several functions, including protecting factor VIII from being digested and cleared from the bloodstream. Some researchers suggest that in doing this, VWF masks some of the sites on the factor VIII protein where antibodies attach, potentially making factor VIII with VWF less immunogenic. Note:

- Intermediate/high-purity plasma-derived factor VIII products are the only ones that contain VWF.
- Recombinant an ultra-high-purity (monoclonal purified) plasma-derived factor VIII products contain no VWF.

Without the protection of its VWF "bodyguard," the immune system may recognize these factor VIII products as intruders and develop inhibitors to neutralize them.

The problem is, no one really knows for sure what causes

inhibitors, and no one knows whether factor VIII with VWF is less immunogenic.

SIPPET STRATEGY

SIPPET set out to answer this question: Is plasma-derived factor VIII with VWF less immunogenic compared to recombinant factor VIII without VWF?

SIPPET researchers designed a study called a prospective randomized controlled trial (RCT). Prospective means looking forward, before the patient has developed an inhibitor (in contrast to retrospective studies, in which researchers look backward, after someone has developed an inhibitor). Controlled means that there are two groups: (1) an experimental group that will use factor VIII containing VWF, and (2) a control group that will use factor VIII without VWF. This second group is used as a standard of comparison against the experimental group. Randomized means that no one involved in the study influenced which group a patient was assigned to. Randomization is often done by a computer.

RCT studies are often considered the gold standard, thought to produce more reliable data than other types of studies. Although an RCT can show relationships between variables being studied, it cannot prove causality. So the RCT used for SIPPET can't prove that the presence or absence of VWF in factor VIII caused the observed results.

SIPPET was conducted between 2010 and 2015, and data was collected on 251 patients from 42 participating sites in 14 countries from Africa, the Americas, Asia, and Europe. The patients were younger than six years old, had severe hemophilia A, were previously untreated with factor, and had minimal exposure (less than five times) to blood components. Of the 251 patients, 125 were treated with one of the plasma-derived factor VIII products containing VWF. The remaining 126 patients were treated with a VWF-free recombinant factor VIII product.⁴ The patients were followed to see if they developed an inhibitor, for 50 exposure days (days they received factor infusions) or three years, whichever came first.

It's important to note that only one of the plasma-derived products used in this study is available in the US, and that the study was funded by manufacturers of plasma-derived products. Is this a conflict of interest? Does it influence the findings?

SIPPET SHORTCOMINGS?

The preliminary findings were startling: of the 251 patients, 76 developed an inhibitor, and 50 of those were high-titer inhibitors. And 90% of these inhibitors developed in the first 20 days of treatment. Most important: recombinant factor VIII products were associated with an 87% increased risk of developing an inhibitor compared to plasma-derived factor VIII products containing VWF.

Remember, these are not final results and have not yet been reviewed by researchers outside of the study. Before you decide whether to switch

The SIPPET Bombshell *continued from page 20*

your toddler to a plasma-derived factor VIII containing VWF, know that many other variables affect inhibitor formation. In any experiment, variables not directly being tested, but which could have an effect on the outcome, are called *confounding variables*.

For example, the single greatest risk factor for developing inhibitors is the type of genetic mutation that caused your child's hemophilia. If the mutation in the factor VIII gene resulted in no factor VIII being produced in his body, then he is already at significantly higher risk of developing an inhibitor. This is one of many confounding variables in the SIPPET study.

One way to reduce the effects of confounding variables on the data is to use a large study sample. If the sample size is large enough and patients are randomly assigned to two groups, then each group should have about the same number of patients with the same confounding variable, so its effect will be canceled. The problem is that the more confounding variables you have, the larger your study sample size must be—perhaps several thousand patients. And many variables affect inhibitor development.

Another way to account for the effects of confounding variables is to identify and measure them, and then to separately compare and analyze the data from patients who share the same confounding variable. This process is called *stratification* (meaning to separate into layers) and was used by SIPPET along with other statistical analysis methods. But the study identified and measured only six confounding variables: (1) age at first treatment, (2) intensity of treatment, (3) type of factor VIII gene mutation, (4) family history, (5) ethnicity, and (6) country site. What about the effects of the other confounding variables that were not measured? If the study sample size was too small to reduce the effects of other, unmeasured, confounding variables, then the study's conclusions are questionable and might be explained in other ways.

DON'T JUMP SHIPYET

At the time of this writing, SIPPET has not been published in a medical journal. That means researchers—outside of those conducting the study—don't know much more about the study than you do after reading this article. Only a short synopsis of the SIPPET study was presented at the ASH annual meeting—just enough to cause a stir and raise many questions. You can be sure that as soon as the journal article is released, it will be examined by bleeding disorder experts worldwide. Questions will undoubtedly be asked about the handling of confounding variables and whether the study sample size was large enough.

And experts will have another question, too: Why didn't the study include any of the new prolonged half-life products, several of which appear to have a lower immunogenicity than other recombinant factor VIII products?

Should you switch your toddler from a recombinant to a plasma-derived factor VIII product containing VWF based on the preliminary SIPPET results, in the hope that it will reduce the

risk of developing an inhibitor? This is a question for you and your hematologist, but if you were a betting person, the answer would be no. To bleeding disorder experts, the results of SIPPET are not a bombshell, but merely a piece of the puzzle that is inhibitors.⁵ The conclusions of this study contradict those of several other studies. It may take years, and several additional studies, to sort everything out. MASAC is on top of this, and as the data becomes available, you can be assured that NHF will share its expert opinion. So keep calm and carry on!

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1. <https://ash.confex.com/ash/2015/webprogram/Paper82866.html> (accessed Feb. 7, 2016).
2. Inhibitors are a major complication of hemophilia in which a person's immune system mistakenly recognizes infused factor as a foreign (and potentially dangerous) protein, and develops antibodies (inhibitors) to inactivate the factor, making factor infusions ineffective.
3. <http://www.hemophiliafed.org/news-stories/2015/12/update-2-sippet-study-2/> (accessed Feb. 7, 2016).
4. The VWF-rich plasma-derived factor VIII concentrates used by SIPPET: Alphanate (Grifols), Fandhi (Grifols), Emoclot (Kedrion), or Factane (LFB). The VWF-free recombinant factor VIII products used: Recombinate (Baxalta), Advate (Baxalta), Kogenate SF (Bayer), or Refacto AF (Pfizer).
5. Visit the Believe Limited website for an excellent interview by Patrick James Lynch of bleeding disorder expert Dr. Steven Pipe about the SIPPET findings: <http://believeitd.com/inhibitors-sippet-and-the-double-edged-internet/> (accessed Feb. 7, 2016).

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Allowing Patients Access to Coverage

By: James Romano

The mission of Patient Services Incorporated (PSI) has been to provide health insurance premium assistance and pharmacy & treatment copayment assistance since our founding over 27 years ago. In 2015 alone, PSI provided assistance to approximately 1,600 families in North Carolina. Helping patients and their families is the guiding principle of our lives every day. Because of our passion for patients, PSI works to remove any roadblocks or challenges patients are facing to receive the healthcare that they need including access to PSI.

Over the last two years PSI has fought an interim final rule issued by the Centers for Medicare and Medicaid Services (CMS) regarding Third Party Premium Assistance for the new plans created under the Affordable Care Act. Through the rule, CMS is allowing health insurance carriers to prohibit premium and cost sharing assistance by charitable organizations like PSI. By giving this authority to insurance carriers, the administration is providing the insurance plans with a back door means of cherry picking expensive patients off of their plans—in essence another pre-existing condition based on medical history and income level. This is unacceptable and disheartening. Plans in the North Carolina Exchange are embracing this prohibition.

I am proud to say the Patient Services Incorporated is fighting to modify this rule so nonprofit charitable organizations can provide assistance to patients. Hemophilia of North Carolina has been a wonderful advocacy partner in this fight. PSI has worked with Congressman Kevin Cramer (R-ND) to introduce H.R. 3742: the Access to Marketplace Insurance Act. This bill amends the Affordable Care Act to include nonprofit organizations, places of worship and local civic groups to the list of entities that health insurance carriers in the federal or state Insurance Marketplaces must accept third party premium assistance. Currently carriers must accept assistance from State Ryan White/AIDS Drug Assistance Programs; Indian Tribes and any other state and federal program.

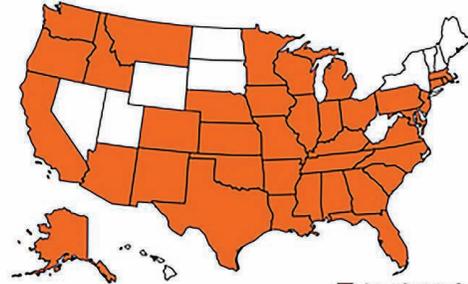
Ultimately, the issue at stake is whether private charities should be able to help Americans, plain and simple. If CMS and the insurance carriers can dictate how a consumer obtains the funds or where the patient can turn to receive help, patient lives will be placed in jeopardy. PSI believes that families should not have to bankrupt themselves obtaining coverage and care—there is no benefit to any entity in the system if that were to happen.

PSI is lucky to have the support of the National Hemophilia Foundation and the Hemophilia Federation of America in this campaign. As of June 10, the legislation currently has 71 cosponsors including Congressman Mark Meadows (R-NC) and Congressman Walter Jones (R-NC). However we need your help. We need the entire North Carolina Congressional Delegation to cosponsor this important legislation. Hemophilia of North Carolina and PSI can work with you to advocate for this legislation with your Member of Congress. If you would like to assist PSI in this endeavor please email me directly at jromano@uneeedpsi.org. We will not stop until this roadblock is removed from the path of patients.

Insurers Across the U.S. are Using a CMS Rule to Deny Coverage to Individuals Who Receive Assistance from Charities

Individuals living with chronic and life threatening illnesses rely on non-profit premium assistance programs to help cover the high costs of their health insurance. Yet, when issuing guidance on acceptable third-party insurance payments for new Exchange plans offered under the Affordable Care Act, CMS failed to include non-profit charitable assistance for patients covered by qualified health plans.

Now, insurance companies in 38 states are citing this rule to deny coverage to patients who receive non-profit assistance, keeping critical treatments and services out of reach for those who need them most.



States with Plans that Have Denied Coverage to Individuals who Receive Non-Profit Premium Assistance

Fortunately, Congress now has an opportunity to put an end to this harmful rule and ensure health care coverage that works for everyone. The Access to Marketplace Insurance Act (H.R. 3742) is bipartisan legislation that would create a carve-out for non-profit charities to provide premium assistance under the CMS third-party guidance.

Let Charities Be Charitable, Co-Sponsor H.R. 3742 Now.



To cosponsor H.R. 3742, please contact Adam Jorde at Adam.Jorde@mail.house.gov

The Marketplace Access Project (MAP) is a patient advocacy movement dedicated to protecting non-profit insurance premium assistance for individuals suffering from chronic and life-threatening illnesses.

www.marketplaceaccess.org

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You can make an online donation to HNC’s 2016 Hemophilia Walk through the National Hemophilia Foundation web site. Just follow these easy steps:

- Go to walk.hemophilia.org/Raleigh
- Click on the blue **DONATE** button.
- Click on “*click here*”.
- Fill in your name, address, and credit card information (all the places marked with “*”). Don’t worry, this is a secure website, so your information is safe.
- Click the blue **Continue** button at the bottom to confirm your donation.

That’s it! You don’t have to “log in” to anything.

You can also use this same website to credit your donation to a specific Walk team or an individual team member. More about that soon!

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- If you shop online, consider using AmazonSmile. AmazonSmile works just like Amazon, but in addition they donate 0.5% of your purchase to the organization of your choice! Consider HNC when making your next purchase!
- Host a garage sale – you can clean out those unused items, and help HNC at the same time!
- Ask for a company match – many companies will work with their employees to match their donations to a charitable organization.
- Serve a meal fundraiser at your local church or community center
- Talk to a local school or business about hosting a raffle





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