

THE HEMO HERALD

A Newsletter of the Brandywine Valley Hemophilia Foundation

Serving the Bleeding Disorder Community of Pennsylvania, Delaware, & New Jersey

BVHF Mission:

Brandywine Valley Hemophilia Foundation was founded in 1972 and is a totally volunteer organization dedicated to serving the Bleeding Disorder communities of Pennsylvania, Delaware, & New Jersey. We provide:

- Education/Outreach meetings & Events for patients & families
- Funding for local Hemophilia Treatment Centers, Hospitals, & Summer camps
- Education scholarships & Conference Travel Scholarships
- Emotional/Financial support for patients/families in times of hardship
- Advocacy in the political spectrum for the continued needs of the Bleeding Disorder community
- Funding for research for improved treatment & the Hope of a Cure



UPCOMING EVENTS:

Due to Covid-19, many of our upcoming in person BVHF events are on hold till further notice. Please see our Webpage for updates, rescheduled dates, & info regarding virtual events!

<http://brandywinehemophilia.org>

**We are ALL in this TOGETHER!
#HEMOSTRONG**



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Amazon Smile, Contact info

Coronavirus (COVID-19) Advice from the CDC

Wash your hands often

- [Wash your hands](#) often with soap and water for at least 20 seconds especially after you have been in a public place, or after blowing your nose, coughing, or sneezing.
- If soap and water are not readily available, **use a hand sanitizer that contains at least 60% alcohol**. Cover all surfaces of your hands and rub them together until they feel dry.
- **Avoid touching your eyes, nose, and mouth** with unwashed hands.

Avoid close contact

- **Avoid close contact with people who are sick, even inside your home.** If possible, maintain 6 feet between the person who is sick and other household members.
- **Put distance between yourself and other people outside of your home.**
 - Remember that some people without symptoms may be able to spread virus.
 - [Stay at least 6 feet \(about 2 arms' length\) from other people.](#)
 - Do not gather in groups.
 - Stay out of crowded places and avoid mass gatherings.
 - Keeping distance from others is especially important for [people who are at higher risk of getting very sick](#).

Cover your mouth and nose with a cloth face cover when around others

- You could spread COVID-19 to others even if you do not feel sick.
- Everyone should wear a [cloth face cover](#) when they have to go out in public, for example to the grocery store or to pick up other necessities.
 - Cloth face coverings should not be placed on young children under age 2, anyone who has trouble breathing, or is unconscious, incapacitated or otherwise unable to remove the mask without assistance.
- The cloth face cover is meant to protect other people in case you are infected.
- Do NOT use a facemask meant for a healthcare worker.
- Continue to keep about 6 feet between yourself and others. The cloth face cover is not a substitute for social distancing.

Cover coughs and sneezes

- **If you are in a private setting and do not have on your cloth face covering, remember to always cover your mouth and nose** with a tissue when you cough or sneeze or use the inside of your elbow.
- **Throw used tissues** in the trash.
- Immediately **wash your hands** with soap and water for at least 20 seconds. If soap and water are not readily available, clean your hands with a hand sanitizer that contains at least 60% alcohol.

Clean and disinfect

- **Clean AND disinfect [frequently touched surfaces](#) daily.** This includes tables, doorknobs, light switches, countertops, handles, desks, phones, keyboards, toilets, faucets, and sinks.
- **If surfaces are dirty, clean them.** Use detergent or soap and water prior to disinfection.
- **Then, use a household disinfectant.** Most common [EPA-registered household disinfectant](#) will work.

<https://www.cdc.gov/coronavirus/2019-ncov/index.html>



BVHF Fall Education Celebration



Please Join Us **September 12th, 4-7pm**

Please join the Brandywine Valley Hemophilia Foundation for a late afternoon gathering including Raw Bar and Hors D'oeuvres, Educational Exhibits and speeches, raffle tickets for prizes. RSVP by August 31st.

Fieldstone Golf Club
1000 Dean Road
Greenville, DE 19807

\$100 per person, this event is for age 21 or older

A discount is available for patients with a bleeding disorder or their caregivers \$60 per person

Refer questions to Board Member and Chairman of this event: Stephen Steinmetz 610-235-1229.



Washington Days—A Teen’s Perspective on Advocacy on Capitol Hill

”How a Teenager Can Make a Difference: My Washington Days Experience”

By: Cole Hamstead, 15yro, Severe Hemophilia A

As I tightened the knot on my red tie, which symbolizes National Bleeding Disorders Awareness Month in March, I am reminded that this is my 10th year attending Washington Days with the National Hemophilia Foundation (NHF). Year after year, I have driven from Delaware to Washington DC with my mom and wear this special red tie. My name is Cole Hamstead and I am 14 years old. Each February/March people from all over the country gather in Washington DC to meet with the senators and representatives from their state to talk about issues that are important to the bleeding disorders community. This year there were over 450 people (wearing red ties) that headed out to Capitol Hill to advocate.

When we arrived on Wednesday afternoon, the NHF had a briefing session to educate us about the issues that we should discuss with our state lawmakers. Nearly every year we advocate for continued funding for the hemophilia programs at the Centers of Disease Control (CDC) and Health Resources and Services Administration (HRSA). Those programs are instrumental in providing funding for the 140+ Hemophilia Treatment Centers across the country. In addition, the CDC is also responsible for surveillance of the blood supply which is important because some of our medications are still derived from blood products. This year we were also seeking support for

a new piece of legislation called the Hemophilia Skilled Nursing Facilities (SNF) Access Act. Oftentimes, people with hemophilia need access to a SNF for additional recovery time after surgery. Most SNFs won't accept Medicare patients with bleeding disorders because the daily payment rate they receive isn't enough to cover the high treatment cost of bleeding disorder's medications. This legislation is requesting that bleeding disorders treatments be added to the list of treatments that SNFs can bill separately under Medicare Part B. We have sponsors for this bill from the Senate and House but now we are looking for others to co-sponsor the act.

On Thursday morning we headed out to Capitol Hill to meet with people from the offices of Senator Coons, Senator Carper, and Representative Blunt-Rochester. Sometimes we get to meet with the Senator or Representative directly and other times we are meeting with someone from their legislative staff that specializes in healthcare issues. My role in these meetings is to talk about what hemophilia is, how it impacts my life, the high cost of my factor medication and the value of my HTC. My mom then talks about the legislation and the hemophilia funding needs. All the people we met with were supportive of the funding for the hemophilia programs at the CDC and HRSA and they also seemed very interested in our Hemophilia SNF Access Act. Our meeting with Senator Coon's office was particularly important because Senator Coons is on the Senate Appropriation's Committee. That is the committee that submits the funding proposal for the annual budget. Each year he has been very supportive in ensuring that our hemophilia programs continue to receive funding. In between our meetings I also enjoyed the chicken tenders and fries at the Senate Cafeteria and snuck in a visit to the Senate Gift Shop!

Friday morning the NHF hosted a session to teach chapters how to advocate within their state. One of the things they told us about was how to obtain a proclamation to make March Bleeding Disorders Awareness Month

in our state. In 2019, I decided to try it and worked with Governor Carney's Office to establish a Bleeding Disorders Awareness Month in Delaware. The Governor had a signing ceremony in his office and all members of the Brandywine Valley Hemophilia Foundation were invited to attend. Having a proclamation like this helps to bring more community awareness to the needs of the bleeding disorders community.

Over the 10 years I have been going to Washington Days, my role has changed. When I first started going, I was there as the face of someone with a bleeding disorder, but I was too young to share my story. As I have gotten older, I have had the opportunity to talk about the healthy active life I have lived so far because of access to the factor medications and treatment centers. I now advocate not just for people like me, but for other people with bleeding disorders that may have needs that are different than mine. I am very aware of how fortunate I am to have the treatments that are available today. I know that it hasn't always been that way and frequently think back to the Ryan White story and the people in the 70s and 80s whose lives were devastated by the tainted factor crisis. Their sacrifice has led the way to changes that make life with a bleeding disorder much better today. There is still much work to be done and participating in Washington Days is one way that I can give back.



Cole at 5 years old on left, and then 15 years old with Senator Carper
10 years of Washington Days Advocacy!
Thank you, Cole for using your voice to support the Bleeding Disorder Community!

BVHF 6th Annual Big RED Run!

Big Red Run 5K-10K Run/Walk



Running & Walking for a Cure

The Big Red Run originally scheduled for May has been postponed!

We hope to race with you in October!

Ridley Creek State Park,
1023 Sycamore Mills Rd.,
Media, PA 19063-4398
Pavilion 17

For more information contact Jill Abrams, Race Director,
610.329.7252, or
jilljillabr@comcast.net



Brandywine Valley Hemophilia Foundation is pleased to welcome our newest Board Member—Christine Rowe!

It is with great pleasure that all at Brandywine Valley Hemophilia Foundation welcomes **Christine Rowe** as our newest Board member. Christine Rowe has strong ties to the bleeding disorder community for the last 17 years. She is the mother of two children, Alex 17 and Courtney 14. Alex has Type 3 VWD and Courtney has Type 1 VWD. Christine also has Type 1 VWD. Alex was diagnosed when he was circumcised at birth. Shortly after, it was important to her to become educated and to advocate for her son. She became involved with the local chapter, organizing many new fundraisers and bringing new external donors to the organization.

Christine began her career working for JP Morgan Chase. Soon her experience in patient advocacy led her to a position within pharmaceuticals working for Biogen, to Bioverativ, and finally to Sanofi Genzyme as the associate director for patient advocacy. While working full time, she also served on the board of directors for the Delaware Valley Chapter and later became the Executive Director for the Foundation.

Recently she completed her MBA and accepted a position as the Director of Patient Advocacy for Alexion Pharmaceuticals. While she no longer works in the hemophilia space, she continues to support the bleeding disorder community and is looking forward to working as a board member of the BVH.

We look forward to drawing on Christine's broad experiences within the bleeding disorder community, both personally and professionally, to the advantage of all the families and friends supported by BVH. All of us look forward to a long and successful partnership!





Research Spotlight: Current Status of New Hemophilia Treatments

By: Chris Ramsey, Hemophilia patient, Hemophilia parent, BVHF Board Member

Over the last few years, the treatment of hemophilia has seen an abundance of new therapies directed at improved prophylactic home therapies and increasing progress toward potential gene therapy “cures”. Many new treatment products have been approved within the last 4-5 years (some as recently as months ago) and are on the market today. Others have submitted applications for FDA approval recently and anticipate decisions later this year.

The first of the new therapies to make it to market were the longer lasting (extended half-life) products. There are now four categories of extended half-life products approved for use in the US: **Fc Fusion** (Alprolix and Eloctate), **PEGylation** (Refixia, Rebinyn, JIVI, Adynovate), **Albumin Fusion** (Idelvion), and most recently **Single-chain** (AFSTYLA). Using one of these longer half-life products in a prophylaxis regime can reduce frequency of injections and increase the minimum factor level maintained.

There are several novel therapies that are in initial research stages, in clinical trial, or have received approval and are available for treatment. HEMLIBRA is probably the best known of these since it has made it to market. Initially released as a treatment for

Factor VIII hemophilia with inhibitors, it is now being prescribed for patients without inhibitors as well. HEMLIBRA is **not** a Factor product. HEMLIBRA is an antibody that was developed to mimic the functionality of Factor VIII: it binds Factor IX to Factor X in the blood clotting activity chain. HEMLIBRA is administered as a subcutaneous injection (not intravenous) and can be dosed as a single injection once per week, once every two weeks, or once per month. Reducing the injection frequency to once per week or less and eliminating the stress of intravenous injections (especially with young patients or patients with inhibitors makes this the most convenient treatment developed so far. HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment.

Fitusiran is an RNA interference (RNAi) therapeutic. It disrupts the targeting of the endogenous anticoagulant antithrombin (AT). The analogy that works the best is to picture a scale, where on one side is a pile of balls that are the procoagulants that are trying to enhance clotting, and on the other side of that scale are the natural anticoagulants that regulate and suppress clotting. Steven Pipe, MD, of the University of Michigan in Ann Arbor, says "Normally our hemostatic system is in a balance between procoagulants and natural anticoagulants. In the hemophilia state you don't have as many balls on the procoagulant side because of factor deficiency, and you still have the full weight of the natural anticoagulants, and that tilts the patient toward a bleeding state". Fitusiran is in phase 3 trials for hemophilia A and B patients with or without inhibitors and is expected to arrive on the market in 2021.

The final entry into new treatments is gene therapy. After decades of research, there is finally a product that is expected to reach the commercial market this year. BioMarin has submitted paperwork to the FDA for final approval of their ValRox, a Factor VIII gene therapy product. ValRox uses a virus shell with the Factor VIII gene inserted into it. A one-time injection of trillions of viral genomes is given. The viral vectors migrate to the liver and begin producing factor in the recipient's liver. There are significant limitations with gene therapy. Some hemophilia patients will already have been exposed to the adeno-associated virus being used as the vector shell and developed antibodies against the virus. Those patients would no longer be candidates for this gene therapy. Cost will be another impediment to gene therapy treatment. BioMarin expects to price ValRox between \$2M and \$3M for the one-time, single shot treatment. Insurance companies may balk at the cost even though the lifetime savings payback for the treatment could be as little as 5-10 years. Several other companies are in Phase three trials of other gene therapy products for treatment of both Hemophilia A and B. Competition in the market may eventually bring cost down, expand the pool of eligible patients, and alter the attitudes of insurance companies regarding payment for gene therapy.

With the proliferation of new and novel therapies, it is an amazing time for hemophilia treatment and the prospect of nearly bleed-free lives.

*****None of this information is intended as medical advice. You should consult your hematologists regarding your treatment and direct any questions regarding alternative therapies to them.*****

Congrats to our BVHF 2020 Scholarship Winner!

Matthew Bayer is the 2020 winner of the Brandywine Valley Hemophilia Foundation's scholarship. Matt graduated from William Tennent High School and Middle Bucks Institute of Technology where he was part of the Web Design and Interactive Media program. He graduated Magma Cum Laude and is a member of the National Honors Society, Science National Honors Society, Mu Alpha Theta and the National Technical Honors Social. He is a young man with serious academic achievements behind him. This fall, he plans to attend Jefferson University, majoring in Graphic Design. Matt hopes to someday successfully combine skills in graphic design with the sports world. All of us at Brandywine Valley Hemophilia Foundation wish him nothing but the best for a successful year amid the confusion of the current pandemic Matt's achievements are hard won and we are pleased to help support him as he moves forward.





The **Hemophilia Federation of America's** Annual Education Symposium has been rescheduled for August 24-29 as a virtual conference.

Find out more via their website.

<https://www.hemophiliafed.org/>



The Amazon Smile logo is displayed on an orange rectangular background. It features the word "amazon" in black lowercase letters with a white curved arrow underneath it, followed by the word "smile" in white lowercase letters. Below this, the slogan "You shop. Amazon gives." is written in black text.

amazonsmile
You shop. Amazon gives.

Do you shop on **Amazon**? Did you know that your shopping on Amazon can help support BVHF without costing you any extra money? Simply register on **Amazon Smile** and choose **Brandywine Valley Hemophilia Foundation** as the organization you want your shopping proceeds to go towards.

Check out our Website updates at www.Brandywinehemophilia.org

Make sure to follow us on Facebook & tag us in any photos from our events!

*If you are interested in having advertising space to sponsor our next Newsletter, contact GVannicola@verizon.net

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