

What should I evaluate when considering participating in a **clinical trial**?



Watch the videos

HEMOPHILIA FORWARD 

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COMMUNIQUE

For Bleeding Disorders

AUGUST 2023

Welcome

The nation's largest bleeding disorder conference took place in Maryland, August 17-20. I wasn't able to join, as I was attending another important event, but I hope it was a great conference. I've attended almost every year (except to have a baby once) since 1992!



There are so many exciting things to learn and share when you attend these meetings. If you couldn't attend in person, you can subscribe to NHF's Notes, which arrive every month, like my Communiqué, in your mailbox. Someone on Facebook complained they didn't get any notice of the upcoming NHF conference: it's up to *you* to be proactive and sign up! You can't help but get notified.

And **kudos to NHF** for eliminating plastic name tags at their events from now on. We need to help our poor planet, even if it is one plastic badge at a time. I hope we can all follow this example at upcoming meetings, and at home.

Stay safe and hopeful,

Laurie

Events

Get Out!

Enjoy the outdoors with a bleeding disorder, in this new series, called Pathfinders. Learn the essentials

about backpacking, hiking and just enjoying nature. Attend a series of 60-minute virtual workshops, free, for the bleeding disorder community. Hosted by the renowned GutMonkey! The first one is August 29, 7 pm EST. To register [click here](#).



Inhibitor Camp for Adults

The Leverage Program is a pioneering national program for young adults from ages 18 and up that have hemophilia with an inhibitor. Life changing experiences allow participants to visit awe-inspiring places and to challenge themselves in novel ways, through outdoor adventure experiential education activities. September 10-14. YMCA Camp Collins, Gresham Oregon. All expenses covered. [Click here for more info](#).



Hemophilia A and the Evolution of Prophylaxis

Register for a free webinar about the standard of care: prophylaxis. In this informative webinar, Octapharma Clinical Nurse Educator Amber Federizo, DNP, will cover the past, present, and future of prophylaxis. She will address potential trade-offs associated with available treatment options, the special considerations for the treatment of women and girls with hemophilia A, and what the future may hold for emerging therapy alternatives [To register click here](#).



For Mature Hemo Bs Only!

The Coalition for Hemophilia B is hosting virtual sessions on aging, especially for those age 50 and over. Topics include: health issues, including spouses' needs; Medicare and insurance; fitness and diet; retirement preparation and more.

Next one is Thursday, August 31. [To register click here](#).



Navigating Hemophilia A: The Patient Journey

Join Claudio Sandoval, MD, resident hematologist at Octapharma, to explore the pain

points, mental health impacts, educational support recommendations, and diagnostic implications associated with each stage of the hemophilia A patient journey. This instructive live webinar event will be followed by an interactive Q&A session. Free. Wednesday, August 30, 8-9 pm EST. [To register click here.](#)



LIVE WEBINAR AND Q&A SESSION

Navigating Hemophilia A: The Patient Journey

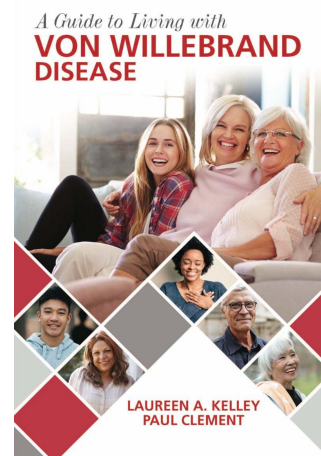
August 30th, 2023 | 8:00 PM EST



Resources

A Guide to Living with von Willebrand Disease is Back!

The world's first book on the world's most commonly inherited bleeding disorder, by Lauren A. Kelley and Paul Clement, is back in print. Topics include learning to cope with VWD, inheritance, the medical system, treatment, women's issues, health insurance. Complete resource guide and real-life stories. This fourth edition was published in 2021. Free to patients, HTCs and nonprofits. Sponsored by CSL Behring. [To order click here.](#)

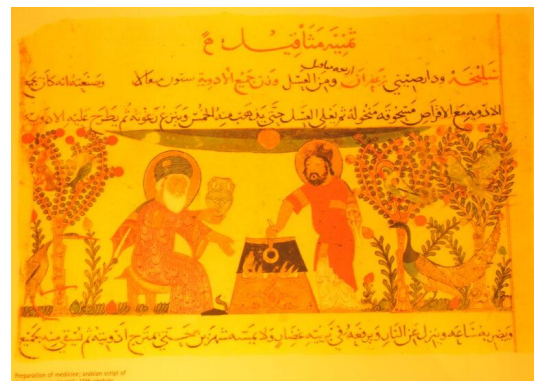


HemaBlog

Origins of Pharmacies

Specialty pharmacies are integral to expert factor delivery. But did you know that the first pharmacies were developed in the 8th century by Muslim scholars?

[Read more about the evolution of pharmacies here.](#)



Advocacy

In For a Bad Surprise

As of 2022, the federal “No Surprises Act” protects people covered under group and individual health plans from receiving surprise medical bills when they get (1) most emergency services (even at out-of-network providers); (2) non-emergency services from out-of-network providers at in-network facilities; and

(3) services from out-of-network air ambulance service providers. People with Medicare and Medicaid already enjoy these protections and are not at risk for surprise billing. [For more info click here.](#)

Medical

New Therapy for Hemophilia B and Inhibitors

Alhemo™ (concizumab injection) is used for hemophilia B patients who have developed inhibitors, which prevent replacement factor IX therapies from working properly. Alhemo contains the active substance concizumab, which belongs to a group of medicines called monoclonal antibodies. The monoclonal antibody in Alhemo recognizes a protein (TFPI) that prevents clotting, and by binding to this protein concizumab helps to increase clotting and stop bleeding in hemophilia patients. It has been approved in Canada, but is still under FDA review in the US. [For more info click here.](#)



Science

Gene Therapy: One Down, But One to Go

Roche is discontinuing its investigational hemophilia A gene therapy called SPK-8016. (SPK refers to Spark Therapeutics, which is carrying out trials) But clinical trials for SPK-8011 are still underway. SPK-8011 aims to deliver a healthy version of the gene that provides the instructions for producing functional factor VIII. SPK-8011, also known as dirloctogene samoparvovec, has been tested previously in a clinical trial to assess its safety and efficacy. Data showed an increased factor VIII activity in 21 of 23 patients, with an average bleeding rates decreased by more than 90% after up to five years of follow-up. SPK-8011 is now moving into Phase 3 testing, according to Roche. [For more info click here.](#)

World

Recycling, Rescuing, Regifting

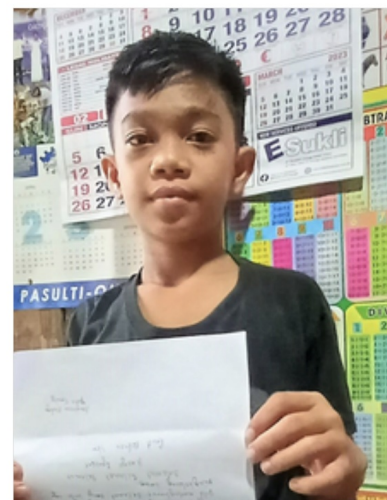
Since March, LA Kelley Communications has received over 2.7 million IU of donated factor, all destined for developing countries. We will soon be announcing a name for our program, which also provides funding for surgeries, prosthetics and more to people with bleeding disorders in developing countries, who are in dire need of assistance, like Abdellah of Morocco. If you have

factor to donate, please contact us first:
laurie@kelleycom.com



August's Child

Efren is 11, and lives in the Philippines. He has moderate hemophilia A, and his parents only earn about \$150 a month. His birthday is November 11. Wouldn't it be great if we could get him a sponsor in time for his birthday? It's only \$35 a month. [To sponsor him click here.](#)



Just in Case You Haven't Heard...

- The global hemophilia therapies market is estimated by one source to be valued at over \$15 billion in 2023 and is forecast to exhibit a continued annual rate of growth of 6.33% from 2023-2030.
- The top manufacturers in the hemophilia marketplace are (in no special order) Takeda, CSL Behring, Biomarin, Pfizer, Bayer, Sanofi; Genentech, Novo Nordisk and Octapharma.
- Other manufacturers include Medexus, HEMA Biologics, and Grifols.

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