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COMMUNIQUE

For Bleeding Disorders

JUNE 2023

Welcome

June is Pride Month, and the bleeding disorder community celebrates our members who identify as LGBTQ+. Summer is upon us, too, and there is a lot happening with fundraisers, community events, hemophilia camps and of course, the Bleeding Disorder Conference in August. I hope you can attend some community events, so welcome after a miserable two years of Covid isolation. Enjoy!

Stay safe and hopeful,

Laurie



Events

Learn about Self-Infusion!

Attend an upcoming, free Factor My Way® live educational webinar on Thursday, June 22nd at 8:00pm EST: "Self-Infusion: Independence, Flexibility, and Control." Presenters Dr. Claudio Sandoval, and Sara Ceresa, mother to twin daughters with VWD, will cover the many

reasons to learn how to self-infuse. Learn about symptom management through self-infusion, ways to take fear out of self-infusing, and practical tips for success by [clicking here](#).

 The image features the Factor My Way logo on the left. To the right is a photograph of a young boy with glasses and a striped shirt, smiling. Below the logo, there is text: "LIVE WEBINAR AND Q&A SESSION", "Self-Infusion: Independence, Flexibility, and Control", and "June 22nd, 2023 | 8:00 PM EST".

B on the Road!

The Coalition for Hemophilia B is taking its meetings to you! Learn about new treatments and connect with other families. In June, meetings take place in Boston, Indianapolis, Birmingham, Salt Lake City and Rochester. [Sign up here](#).



Resources

Compare and Contrast!

Overwhelmed by all the choices in factor

products? Download our Factor Comparison Chart and see your choices by manufacturer, class of product, half-life and more. [Find it here.](#)



Camp Ho!

Camp is more than just a fun time. It's essential to children's growth, socialization and even learning to infuse. [Check HFA's list of camps](#) for kids with bleeding disorders to see if one is near you.

New MASAC Documents Ready

You can read the latest announcements by NHF's Medical and Scientific Advisory Committee (MASAC) in documents 275, 276, 277 and 278. They cover information about physical therapy, gene therapy, vaccines and more. Search for them and [download here.](#)

HemaBlog

June is National Pride Month, and we are proud to have published the earliest if not the first article on the LGBTQ community by a community member. [Read about it here.](#)



Advocacy

The Power of Pharmacy Benefits Managers

Pharmacy benefits managers (PBMs) are more than just middlemen that create and administer drug formularies on behalf of health insurers, self-insured employers, Medicaid and other payers. They are among the most powerful forces in insurance today—and can affect your access to factor. Lawmakers in May held multiple hearings into the role that PBMs play in our health care system. [Read about it here.](#)



Tracking State Medicaid Disenrollment

Over 758,000 people from 14 states have been taken off Medicaid since April. Over 249,00 were from Florida alone. Kaiser warns as many as 24 million people could lose Medicaid coverage by year's end. [Learn more here.](#)

Medical

Where's my Stimate?

In July 2020 Ferring Pharmaceuticals halted manufacturing of the nasal spray Stimate®, a used to treat bleeds in mild hemophilia A and some types of von Willebrand disease. The suspension and subsequent recall were in response to a packaging seal issue that caused

evaporation, a potential hazard to patients who could be exposed to dangerously high concentrations of the product's active ingredient desmopressin acetate. [Read NHF's article here to learn more.](#)



Science

Can Gene Therapy be Better?

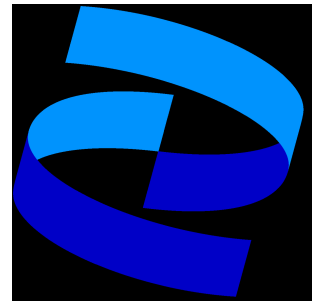
The long-awaited cure finally has arrived. But how good is it? One article reviews concerns about gene therapy held by different specialists. “These therapies are also quintessentially first-generation, and investigators see room for improvement.” [Read the article here.](#)

Sub-Q Hemophilia B Therapy Fast-Track

Centessa Pharmaceuticals announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to SerpinPC, an investigational subcutaneously administered novel inhibitor of activated protein C (APC) being developed for the treatment of hemophilia B, with or without inhibitors, which may also be developed to prevent bleeding associated with other bleeding disorders. [Learn more here.](#)

Good News from Pfizer

Pfizer announced that the pivotal Phase 3 BASIS clinical trial evaluating *marstacimab* has demonstrated statistically significant and clinically meaningful effects. Marstacimab, a novel, investigational anti-tissue factor pathway inhibitor (anti-TFPI) being studied for the treatment of hemophilia A or B for people without inhibitors to factor VIII or factor IX, was administered weekly with flat (not weight-based) dosing in the trial as a subcutaneous 300 mg loading dose followed by 150 mg once weekly. [For more info click here.](#)



World

June's Child

Salmanfaris is 12, and his birthday is December 10. He lives in the southern part of India, where it is lush and hot. He lives in poverty and needs assistance. For only \$35 a month, you can change his life! [To sponsor him click here.](#)



Read What the WFH Did in 2022!

The World Federation of Hemophilia had major successes in 2022: reaching 25,000+ people with bleeding disorders, supporting 115 treatment centers and reaching 156 countries. [Read more here.](#)



Just in Case You Haven't Heard...

- A **central venous access device** (CVAD) is an implanted medical device used for IV infusions without having to access a vein. CVADs include internal devices, called ports, and external devices, called central lines.
- **Anaphylaxis** is a severe, potentially life-threatening whole-body allergic reaction, sometimes occurring after a factor infusion. Although anaphylaxis is rare, people with hemophilia B with inhibitors are at significantly higher risk of anaphylaxis if infused with factor IX.
- **Acquired hemophilia** is a rare and life-threatening autoimmune disorder in which the immune system mistakenly attacks factor VIII. It's often triggered by an illness such as cancer, and usually occurs in the elderly and sometimes in post-partum women.

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