

There are ways to help you

AFFORD YOUR HEMOPHILIA A MEDICINE.

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COMMUNIQUE

For Bleeding Disorders

OCTOBER 2023

Welcome

A client new to the bleeding disorders community recently wanted more information about hemophilia; where could they go? Ten years ago there were not many options on line; I was happily handing out copies of my book. But now? Educational resources are abundantly available at the touch of a computer key; it's almost overwhelming!

And books have not disappeared. My own *Raising a Child with Hemophilia* will be re-released soon, and we have a new book in the community! Read below. And get smart!

Stay safe and hopeful,

Laurie



Events

Learn About Gene Therapy

Join BioMarin Pharmaceutical for a virtual event to learn about one-time Roctavian gene therapy for eligible adults with severe hemophilia A. Hear from a person with severe hemophilia

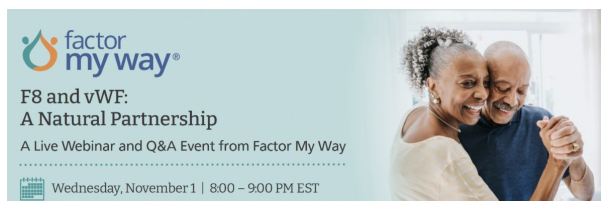
A to learn about their Roctavian experience, learn about eligibility testing, and get answers to your questions. Roctavian is a one-time gene therapy used for the treatment of adults with severe hemophilia A who do not have antibodies to the virus, AAV5 which is determined by a blood test. November 1, 7:30 pm EST. [To register click here.](#)



Perfect Pair: Factor VIII and VWF

Why is von Willebrand factor so important for determining factor VIII levels? Attend a free Factor My Way® live educational webinar called "F8 and vWF: A Natural

Partnership" on Wednesday, November 1 at 8:00 pm EST. [To register click here.](#)



Last Chance for Adventure!

The final virtual session of Pathfinders is on October 18, 7 pm

EST. Don't miss out! Learn the essentials about backpacking, hiking and just enjoying nature. This 60-minute virtual workshop is free, and designed for the bleeding disorder community. Hosted by GutMonkey. [Log in here at 7 pm EST.](#)



Resources

VWD Book for Children!

Fun Facts About a von Willebrand Warrior is a new softcover storybook for children with von Willebrand disease that empowers and educates children about their disorder in a positive format. To order email: Jcesta@VWDCConnect.org



Help is Here

“Healthy Living Items Assistance” helps people with bleeding disorders defray the cost of medically necessary items that are not covered by insurance, or even by out-of-pocket payment. This includes wheelchairs, crutches, splints, and even workout equipment to help you live a healthier life! [To apply click here.](#)

HemaBlog

The Parent-Teacher Relationship

It's normal for anyone to be concerned at the thought of a child with hemophilia in the classroom, but it can especially worry the teacher, who bears responsibility for the students. Even if the teacher has some knowledge or experience, no two children with hemophilia are alike. Chances are, yours will be the first child with hemophilia that his teacher has met. What does the teacher need to know? [Learn more here.](#)



Advocacy

Know Who Represents You

The United States Congress has two chambers, one called the Senate and the other called the House of Representatives (or “House” for short) which share the responsibilities of the legislative process to create federal statutory law.

They occupy opposite ends of the Capitol Building. Use GovTrack to find out who represents you in Congress, what bills they have sponsored, and how they voted. This is a great tool for when you need to make your voice heard regarding anything about bleeding disorders. [For more info click here.](#)

Medical

Survival Disparity in Hemophilia

Survival rates of people with hemophilia have improved through the years, but the median age of death is consistently ten years lower for Black and Hispanic males compared to White males. This disparity warrants further research, say the authors of “Racial and ethnic differences in reported haemophilia death rates in the United States,” published in the September issue of the journal *Haemophilia*. [Read it here.](#)

Help for People with Substance Abuse

National Bleeding Disorders Foundation (NBDF) has published Document 279, “Recommendations on Reducing Barriers to Substance Use Disorder (SUD) Treatment Facilities and Programs for Persons with Bleeding Disorders (PWBD),” in response to reports of denied access to residential substance treatment facilities to people with bleeding disorders. The document provides an overview of medical needs related to treatment and management of hemophilia, including the critical role of HTC’s. The document gives clear parameters for what does and doesn’t constitute medical stability from a bleeding disorder perspective, encompassing key factors such as treatment protocols and adherence, patient autonomy, and bleeding event scenarios. Document 279 is a helpful tool for advocating for fair consideration when it comes SUD access on behalf of PWBDs. [Download here.](#)

Bypassing Agents Explained

Learn about bypassing agents as treatment for inhibitors and whether they might be right for you. [For more info click here.](#)

Science

New Inhibitor Drug Investigated

The U.S. Food and Drug Administration (FDA) has granted orphan drug designation to Baudax Bio’s investigational T-cell therapy TI-168 for the treatment of hemophilia A with inhibitors. TI-168 is designed to train the immune system to lower its response to FVIII replacement therapies, to reduce the production of inhibitors. TI-168 uses regulatory T-cells (Tregs), a type of immune cell that helps suppress immune responses from other cells and prevent the immune system from mistakenly attacking healthy tissues. Phase 1/2a clinical trial of Treg therapy planned for early 2024. [For more info click here.](#)

Baudax BIO[®]

Pfizer’s Latest

Marstacimab (PF-06741086) is under development for the treatment of hemophilia A and hemophilia B. It’s administered through intravenous or subcutaneous route. It is a monoclonal antibody that targets tissue factor pathway inhibitor (TFPI). The study demonstrates statistically significant and clinically relevant reduction in annualized bleeding rate compared to prophylaxis and on-demand intravenous regimens. Marstacimab, if approved, has the potential to become the first once-weekly subcutaneous treatment for people with hemophilia B and the first treatment administered as a flat dose for people with hemophilia A or B. [For more info click here.](#)

World

Doug Did It!

Doug Mildram successfully completed a cross-country, 3,784 mile bicycling marathon over six and a half weeks, to raise money for Save One Life. His epic journey honors Barry Haarde, first known person with hemophilia to cycle across America (six times). The fundraiser garnered over \$230,000 to support the mission of the nonprofit, which supports over 1,200 children and young adults with direct sponsorships; it also supports summer camps, offers scholarships and microenterprise grants, and supplies millions worth of factor annually. [Read more here.](#)



October's Child

Kushal is an 8-year-old who lives in Pune, India. He has hemophilia A, and his parents earn only about \$200 a month. He's been waiting for a sponsor, who can augment the family income. Sponsorship is only \$35 a month. Can you help? [To sponsor click here.](#)



Just in Case You Haven't Heard...

- Versiti has launched its new Anti-von Willebrand Factor (VWF) Antibody Assay, a first-of-its-kind assay designed to assist clinicians in identifying patients suspected of having acquired von Willebrand disease.
- A patient in Germany with severe hemophilia A was treated with Rocatavian, marking the first time that the recently licensed gene therapy has been provided commercially in Europe.
- Roctavian is now also commercially available in the U.S., and HTC's have begun screening people with severe hemophilia A to determine eligibility.

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