

#MyHemophilia Truth

Real stories. Powered by sanofi

It's time doctors consider  
your whole experience of  
managing hemophilia.

Change the Conversation

sanofi

© 2023 Genzyme Corporation. All rights reserved. Sanofi is a registered trademark of Sanofi or an affiliate. MA7105-2307634-v1.0 10/2023

LA KELLEY  
communications, inc.

# COMMUNIQUE

For Bleeding Disorders

MARCH 2024

## Welcome

March is Hemophilia Awareness Month but it seems to be gene therapy awareness month. We've got good news and sad news. Sad news is that only one patient last year bought the new gene therapy. The good news is that we have a bright future ahead, with more gene therapy in the pipeline. Even India is getting in on it! It all means that we've got to increase our advocacy efforts like never before, to pressure insurance and the government to look at the economics of covering this expensive disorder. Gene therapy should be at least one viable option—and treated as one with coverage.



Stay safe and hopeful,

Laurie

## Events

### Inhibitor Camp Registration Open

Registration for "After the Shock" opens March 29. See if your child age 0-18 with hemophilia and an active inhibitor are qualified to attend this fabulous family camp from June 21-24. Funding provided for those selected to attend. [Learn more here.](#)



### WFH 2024 World Congress

The largest international gathering dedicated to global issues of hemophilia takes place April 21-14 in Madrid, Spain and is hosted by the World



**WFH 2024**  
**WORLD**  
**CONGRESS**

Federation of Hemophilia. [Learn more here.](#)

## Hurry! HFA is Almost Here!

Attend Hemophilia Federation of America's 2024 Symposium, April 11-13, in Indianapolis! It's one of the largest, family-friendly events for members of the bleeding disorders community. The three-day symposium offers: sessions on advocacy, pain management, addiction, parenting, and women and girls, and more; education for rares, milds, severes, and moderates in both English and Spanish; rap sessions for blood sisters, blood brothers, families, Sangre Latina, milds and spouses and partners; and an excited Final Night Event! [Register here.](#)



---

## Resources

### Be Social!

Our advocacy groups have many social media platforms. Try these for HFA:

YouTube: <https://www.youtube.com/c/HemophiliaFederationofAmerica>

X (Twitter): [twitter.com/hemophiliafed](https://twitter.com/hemophiliafed)

LinkedIn: [www.linkedin.com/company/hemophiliafederationofamerica/](https://www.linkedin.com/company/hemophiliafederationofamerica/)

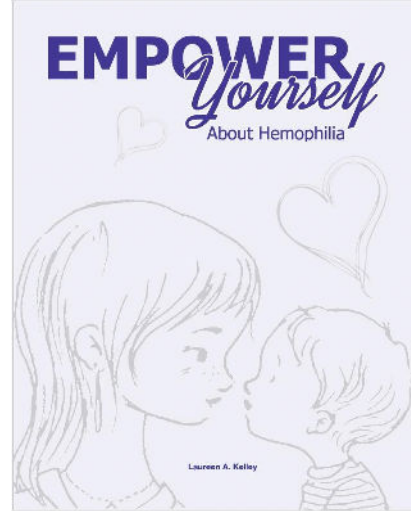
Facebook: [www.facebook.com/hemophiliafed](https://www.facebook.com/hemophiliafed)

Instagram: [www.instagram.com/hemophiliafed/](https://www.instagram.com/hemophiliafed/)



### Think Positive!

A hemophilia diagnosis can upset your beliefs, feelings, and life. This book offers simple, concrete ways to regain feelings of control through empowering ways of thinking and goal-setting. Before-and-after illustrations highlight the many situations you may face, and how to handle them confidently and effectively. Free to families and patients. S&H applies. [Available in paperback or eBook on our website.](#)

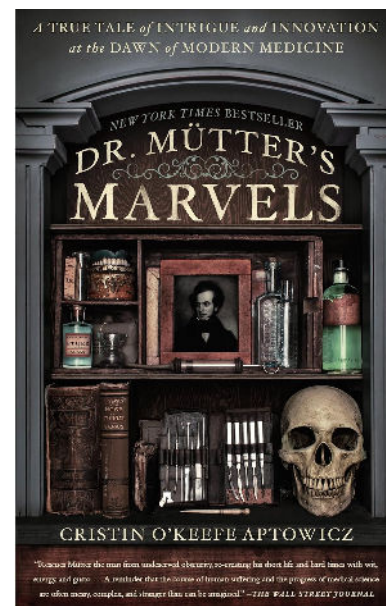


---

## HemaBlog

### Dr. Mütter's Masterpieces

He transformed people's faces by introducing plastic surgery into the United States. A pioneer of medicine, Thomas Mütter can teach our community much about advocacy and patient-centric medical care. [Read why here.](#)

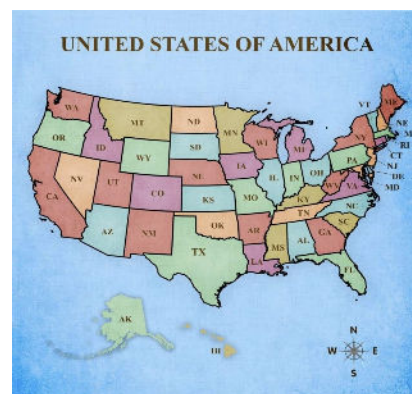


---

## Advocacy

### State of the States

HFA published its State of the States recently. Highlights include: The New York governor signed legislation to strengthen consumer protections against step therapy (fail-first) protocols by health plans. Legislation to protect health plan consumers from copay accumulator programs (CAAPs) is almost approved in Missouri and Oregon, but is still pending in at least six others (including California). A solid 19 states (plus DC and Puerto Rico) have already acted to ban CAAPs in state-regulated health plans. [For more info click here.](#)

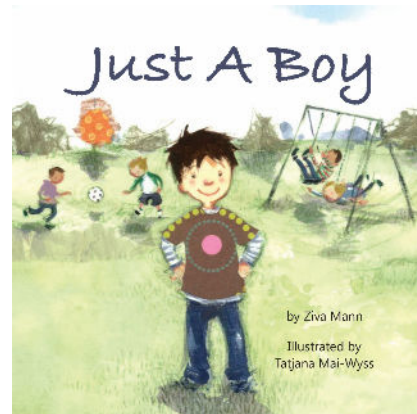




## Medical

### Fear of Needles?

A phobia to needlesticks is a real thing. [Read a great article here](#) about how one father helped his son with hemophilia overcome his fear. Also [request a free copy](#) of *Just a Boy*, to help your child transition from ports to peripheral sticks.



### New Device Can Diagnose Hemophilia Fast

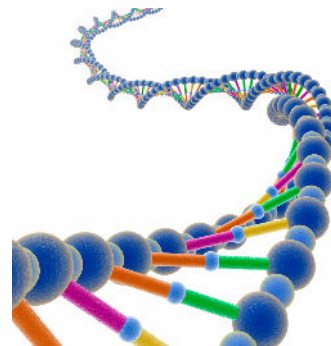
A trio of researchers at Chung Yuan Christian University, Taiwan developed a device that can rapidly determine both blood type and whether someone has hemophilia A. This has important implications in emergencies, when blood transfusions are required. [Read more here.](#)

---

## Science

### Gene Therapy Struggles in Market

Although it was eagerly anticipated, commercial sales of gene therapy are disappointing. BioMarin's hemophilia treatment Roctavian had only one US patient last year. And more gene therapy products are in the pipeline from competitors. [For more info click here.](#)



### But Clinical Trials Continue

Intellia Therapeutics and Regeneron will begin mid-year the first-in-human trial testing of a CRISPR/Cas9-based factor IX gene-editing therapy for people with hemophilia B. CRISPR/Cas9 uses short pieces of RNA designed to target a specific part of a gene, such as FIX. The RNA binds to the gene, then triggers an enzyme called Cas9 to cut the DNA. Then, the cells' DNA-repairing process activates to repair the gene. It works by correcting the mutated gene or inserting a healthy copy of the gene into patient cells. [For more info click here.](#)

---

## World

### Canada Considers Gene Therapy

An excellent article explains how Canada's government is considering paying \$3-4 million per

person for gene therapy, and why living successfully with hemophilia depends on where one is born. [Read here.](#)



## India in Gene Therapy Race

Scientists at the Christian Medical College (CMC) Vellore (in south India) have successfully conducted the country's first human clinical trial of gene therapy for hemophilia A. The clinical trials involved deploying a novel technology of using a lentiviral vector to express a FVIII transgene in the patient's own hematopoietic stem cell, which will then express FVIII from specific differentiated blood cells. India is home to the second-largest population of patients with hemophilia, an estimated 136,000.

---

## In Case You Haven't Heard...

- Gene therapy is the use of genetic material to treat or prevent disease. Research into gene therapy for hemophilia has been underway since the 1990s.
- The goal of hemophilia gene therapy is to get a working copy of a gene into the body so the body can then produce functional factor VIII or IX on its own.
- Gene addition is the most successful and most practical type of gene therapy, which involves adding a new, functional gene. Most hemophilia gene therapy clinical trials involve gene addition.

---

LA Kelley Communications, Inc.  
[kelleycom.com](http://kelleycom.com)



LA Kelley Communications, Inc. | PO Box 846, Georgetown, MA 01833

[Unsubscribe amanda@kelleycom.com](mailto:unsubscribe@kelleycom.com)

[Update Profile](#) | [Constant Contact Data Notice](#)

Sent by [laurie@kelleycom.com](mailto:laurie@kelleycom.com) powered by



Try email marketing for free today!