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For Bleeding Disorders

## Welcome

A new year and a new era in hemophilia, as we welcome our first ever FDA-approved gene therapy for hemophilia B. For those of us with children with hemophilia born in the late 1980s, this is what we have been waiting for. It will be vital for you as a patient or parent, health practitioner or chapter leader, to read more, and learn about when and why this would be an option for patients. It comes with a price, and we will need to continue advocating for our choice of therapies.



Scroll below too, to see events, webinars, new resources and more!

Stay safe and hopeful,

Laurie

## Events

### Have Some Porpoise!

Come see Hemophilia Federation of America! Join friends, family and experts in the bleeding disorder community in person at SeaWorld, Florida, April 13-15, 2023. Details to come. [Click here for more info.](#)



## Programs

### Register for Our Paid Surveys

LA Kelley Communications has partnered with MedPanel, Inc., a full service market recruitment agency specializing in chronic diseases and disorders to conduct surveys, interviews and focus groups to learn what bleeding disorder patients experience. If you haven't already, register now to take advantage of our paid surveys, ongoing. Email: [laurie@kelleycom.com](mailto:laurie@kelleycom.com)

### Be a Lady! (Bug)

Register for the Winter Webinar series with LadyBugs, a program, designed to educate and foster community for women with bleeding disorders. Topics include Reproductive Health, ReBalancing the Body with Diet and more.

[Register now for the January 26 opening](#)

[session.](#)



### Podcast: Interview with NHF's CEO Dr. Len Valentino

In addition to being CEO, Dr. Valentino was a fantastic and beloved pediatric hematologist at RUSH University Hospital. Here, Bloodstream interviews Dr. Valentino's first years as NHF's CEO. [Go here to listen.](#)



### Factor My Way Webinars

Although we just missed a great webinar on nosebleeds with an expert, you can sign up for future ones. These live webinars include a Q&A with the guest expert. Sponsored by Octapharma. [Register here.](#)

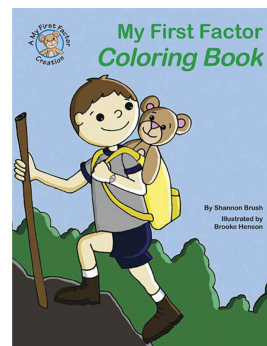


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## Resources

### Coloring Hemophilia

Do you have a little one who likes to color? We still have free copies of My First Factor Coloring Book. This book is a companion to the popular My First Factor series. [Order here.](#)



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## HemaBlog

### Popular Podcasts

Do you like to listen while you drive, workout, clean the house, infuse? Here is a rundown of some popular podcasts in the hemophilia community. [Check it out here.](#)



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## Advocacy

### A Capitol Idea!

Washington Days is in-person this March! Don't miss this important opportunity to share the advocacy needs important to you with your elected official. Registration closes on Feb. 4, so [sign up soon here!](#)



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## Medical

### Gene Therapy for Hemophilia B Approved!

The first gene therapy for adults with hemophilia B —Hemgenix (*etranacogene*

dezaparvovec) is commercially available. A one-time treatment, Hemgenix is expected to prevent bleeds while allowing patients to skip or stop their prophylactic therapy. Hemgenix is indicated for those who are currently on prophylactic therapy or have (or have had) life-threatening bleeding or repeated, serious spontaneous bleeding episodes. It's cost is estimated at \$3.5 million. [For more info click here.](#)

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## Science

### Clinical Trial Needs You!

Researchers looking for a better way to treat people with hemophilia A need participants in a clinical trial. The main purpose of this study is to learn how well a new scoring approach works to select a treatment plan for the prevention of bleeding in people with hemophilia A who switch their treatment from standard half-life products to Jivi®. Different types of information will be used to calculate the risk score like bleeding history, certain biological factors, or physical activity of the participant. All participants will receive Jivi for 6 months. [For more info click here.](#)

### More Gene Therapy in the Pipeline

Long-term data reinforces one gene therapy's potential for hemophilia A. A one-time treatment with Spark Therapeutic's investigational gene therapy SPK-8011 led to sustained low bleed rates for people with hemophilia A in a phase 1/2 clinical trial, with 21 patients experiencing a 92% drop in average annual bleed rate (ABR). [For more info click here.](#)



### Positive Gene Therapy Results

Pfizer had positive results from the Phase 3 BENEENE-2 study evaluating *fidanacogene elaparvovec*, an investigational gene therapy, for the treatment of adult males with moderately severe to severe hemophilia B. The results showed a mean ABR of 1.3 for the 12 months from week 12 to month 15, compared to an ABR of 4.43 during the lead-in pre-treatment period of at least six months, resulting in a 71% reduction in ABR. [For more info click here.](#)

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## World

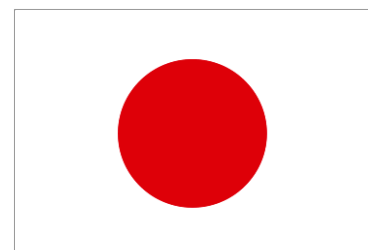
### January's Child

Each month we will feature a child in need of sponsorship. Sankar is 15, and has severe hemophilia A. He lives in India, where his family earns only \$100-150 a month. Life is so hard for him and his family! Can you help sponsor him? For only \$35 a month, you can change his life! [For more info click here.](#)



### Japan's Clinical Woes

Although hemophilia A patients in Japan are using more factor VIII replacement therapy, even prophylactically, they are still the same rate of hospitalizations for complications, according to a recent study. Japan has the third largest economy in the world. [For more info click here.](#)



## Just in Case You Haven't Heard...

- BioMarin's Biologics License Application (BLA) for *valoctocogene roxaparvovec* was accepted for review by the US FDA with a review action date of March 2023.
- Pakistan will issue its first-ever hemophilia treatment guidelines.
- von Willebrand disease is the most common, inherited bleeding disorder.
- Hemophilia B is also called Christmas disease, named after Stephen Christmas, the first patient correctly diagnosed with it in 1952.

