

COMMUNIQUE

For Bleeding Disorders

JULY 2023

Welcome

Talk about fireworks! And not from the Fourth of July. I mean from all the news on gene therapy. Read below: we already have gene therapy for hemophilia B, but at long last, we now have FDA approval for a gene therapy for hemophilia A, which affects 85% of those with hemophilia. The more you know, the better you can decide which therapy is right for you.

Stay safe and hopeful,

Laurie



ADVERTISEMENT

An advertisement for Sanofi. It features a man in an orange shirt looking up and to the right. The text reads: "See hemophilia in a new light." Below this is a blue button that says "LEARN MORE". At the bottom, the Sanofi logo is displayed. Small text at the very bottom reads: "© 2023 Genzyme Corporation. All rights reserved. MAC-US-2302506-v1 3-04-2023".

Events

Joint Health Conference

A two-day, virtual event dedicated to educating and empowering people to prioritize joint health. Expert speakers, interactive workshops, and practical tips will provide valuable knowledge and strategies to enhance joint strength and mobility. September 26-27. Hosted by Hope Charities. Registration to be announced.



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.](#)



Menopause: Expect the Unexpected

Join a clinical nurse from Octapharma during a webinar that reviews what menopause is, how it happens and what to expect as you, a woman with a bleeding disorder, enter this phase of life. July 19, 8 pm EST. [Click here to register.](#)



The Silver Hemo Bs

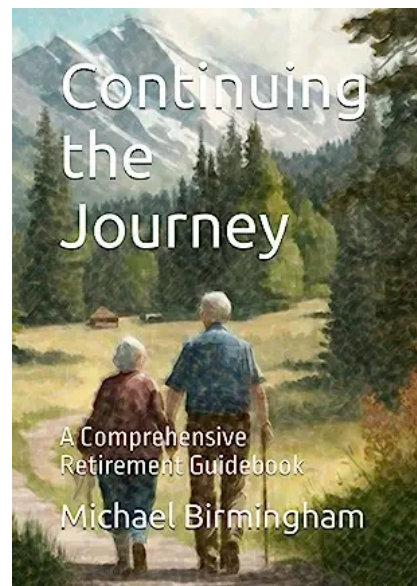
The Coalition for Hemophilia B is hosting virtual sessions on aging, especially for those age 50 and over. Topics include: health issues, including spouses' needs; Medicare and insurance; fitness and diet; retirement preparation and more. Thursdays, now through November 9, 2023. [Click here to register.](#)



Resources

New! Retirement Guide for Bleeding Disorders

Continuing the Journey by Michael Birmingham, a man with hemophilia, is the essential retirement guidebook. It provides comprehensive knowledge and practical strategies to navigate retirement planning and living. Step-by-step guidance on creating a tailored retirement plan, covering financial aspects such as retirement savings, investments, and Social Security. It also explores the psychological and emotional aspects of retirement, with advice on life purpose, relationships, and personal growth. [Click here to order.](#)



Gene Therapy Explained

Pfizer has an interactive website that explains how gene therapy can work to reduce bleeds and frequent infusions. The site shows a futuristic city, where, with a click, the viewer can open up different buildings to learn more. The site contains a glossary of terms, frequently asked questions about gene therapy, the science behind gene therapy, and a sign up form if the viewer wishes to stay connected to learn more. [To participate click here.](#)



Clinical Trials: Want to Participate?

The Hemophilia Federation of America (HFA) has created a clinical trial finder to help people with hemophilia and other bleeding disorders find clinical studies that they might like to participate in. The site allows visitors to search across all clinical trials, regardless of sponsor, for key information and eligibility requirements. It also includes information about clinical trial types, phases, and things to consider before agreeing to participate. The trial finder, powered by Citeline Connect, links directly from the HFA website. [For more info click here.](#)



HemaBlog

Co-Pay Caveats

A two-part series on threats to co-pay assistance was published June 25 and July 2 on HemaBlog, authored by Paul Clement. The articles explain what co-pays are, which co-pay assistance programs exist to help offset the cost of high-priced drugs, and how insurance companies seem determined to keep the benefits. [Read to learn what you can do to manage your expenses](#)



Advocacy

Will You Lose Your Medicaid?

Medicaid disenrollments are now underway in about 40 states. Early reports show that the Medicaid "unwinding" is causing worryingly high rates of coverage losses. [Read about HFA's efforts to protect coverage](#)

Medical

First Hemophilia A Gene Therapy

Approved

The US Food and Drug Administration (FDA) approved Roctavian, a one-time gene therapy for adults with severe hemophilia A who do not have antibodies to the AAV5 virus. Roctavian uses a modified virus, called a vector, to deliver a working copy of the factor VIII gene to liver cells to enable the body to produce its own factor VIII. The vector does not contain viral DNA and does not cause disease. Its safety and efficacy was evaluated in a multinational study in adult males aged 18 to 70 years with severe hemophilia A, who had previously been treated with factor VIII replacement therapy. After the infusion, the mean annual bleeding rate decreased from 5.4 to 2.6 bleeds per year. Most patients were given corticosteroids to suppress the immune system so that the gene therapy would be effective. [For more info read here.](#)



World

July's Child

Muni is only 7, but knows the pain of someone who has lived much longer. He lives in the southern part of India, and his birthday is September 29. He lives in poverty and is waiting for a sponsor to change his life. It's only \$35 a month. [To sponsor him click here.](#)



Just in Case You Haven't Heard...

- Gene therapy is not for children: their livers are rapidly growing and since the added gene is not part of the cell's genome, it is not copied when the cell divides, and the effect of the gene therapy is rapidly diluted as the liver grows.
- Gene therapy doesn't really cure hemophilia; it can change someone's bleeding patterns from those like someone with severe hemophilia, to someone with mild hemophilia.
- Gene therapy works with only one shot.



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