



Special Edition

All About Gene Therapy

With the FDA approval of gene therapies for both hemophilia A & B, we thought it would be good to create a special edition newsletter to help shed some light on these new treatments. All of the information provided is for educational purposes only and shouldn't be taken as medical advice. We don't recommend any specific treatment option, because what works is different for everyone. No matter what your treatment path entails, it should always be a discussion between you and your hematology care team.

Let's Get Started

What is gene therapy? The simple definition: it's a treatment where working genes are introduced into a person's cells to fight disease. For people with hemophilia, gene therapy introduces genes that know how to make factor. It's not a cure, but it can make a lasting, life changing impact.

NBDF's President & CEO, Len Valentino, put on a half hour Gene Therapy 101 webinar specifically for the patient audience. If you are new to gene therapy, it's a great place to start. Watch it [HERE](#).

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An advertisement for HEMGENIX. The background is a gradient of purple and teal. On the right side, there is a large, 3D-rendered DNA double helix structure. On the left side, the HEMGENIX logo is displayed, consisting of a stylized DNA helix icon followed by the text "HEMGENIX®" and "etranacogene dezaparvovec-drlb" below it. Below the logo is a QR code and the text "LEARN MORE AT HEMGENIX.COM". At the bottom left, there is small text: "HEMGENIX is manufactured by uniQure Inc. and distributed by CSL Behring LLC. HEMGENIX® is a registered trademark of CSL Behring LLC. ©2023 CSL Behring LLC, 1020 First Avenue, PO Box 61501, King of Prussia, PA 19406-0901 USA www.CSLBehring.com www.HEMGENIX.com USA-HGX-0393-MAR23". At the bottom right, the "CSL Behring" logo is shown in red.

Is Gene Therapy Right for You?

You may be wondering if you'd be a good candidate for gene therapy. Of course, this is a discussion you'll have to have with your hematology care team, but there are some guides to help you get started.

This first resource is the [Shared Decision Making Tool](#) from the World Federation of Hemophilia. This is an interactive guide that goes step by step to help you assess your goals, treatment options, and who you should involve in the conversation about your treatment options. It's really easy to understand and work through.

This second resource is more like a big handbook of gene therapy FAQs put together by an international and multidisciplinary group known as the Council of the Hemophilia Community (CHC). [The handbook](#) lists the steps of gene therapy from the pre-treatment phase through the follow up that may take place several years after the treatment.

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OUR VISION: A WORLD WHERE NO LIFE IS LIMITED BY GENETIC DISEASE

At Spark® Therapeutics, we are committed to discovering, developing and delivering gene therapies.

We believe investigational gene therapy has the potential to be transformative in the treatment of hemophilia and we understand the importance of developing gene therapies that meet the needs of the hemophilia community. Our priority is the safety and well-being of clinical trial participants.

EXPLORE THE SCIENCE OF GENE THERAPY RESEARCH

Be informed and feel empowered when you learn about the field of gene therapy and its potential application for hemophilia.

Discover what gene therapy is meant to do.
[Visit HemophiliaForward.com >>](#)

Learn about gene therapy research for hemophilia.
[Hemophilia gene therapy guide](#)

Interested in enrolling in a Spark-sponsored hemophilia gene therapy clinical trial?
[Spark clinical trial information](#)

Want to know more about gene therapy clinical trials?
[Clinical trial FAQs](#)

See a demo about the science of gene therapy.
[Watch video >](#)

Explore frequently asked questions about hemophilia gene therapy clinical trials.
[Clinical trial FAQs >>](#)

SPARK THERAPEUTICS

HEMOPHILIA FORWARD

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Clinical Trials

You may also be interested in learning more or participating in a gene therapy clinical trial. Clinical trials allow people to participate in research that answers questions about emerging treatments. There are pros and cons to participating in any clinical trial. It's important that you speak to your hemophilia care team to make sure that you are fully aware of what those pros and cons are before you agree to being part of a clinical trial. For more info on the clinical trial process, check out [this article](#) sponsored by Spark Therapeutics.

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We're Listening

At Pfizer Hemophilia, we have always been deeply committed to you and to listening to what you have to say. Over the years, what you've shared with us has proven invaluable. The events we sponsor, the technology we develop, and the educational materials we create are all designed in response to the requests, needs, and desires of the hemophilia community.

We are grateful for having the chance to partner with you.

—Your Pfizer Hemophilia Team

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More Resources

The National Bleeding Disorders Foundation recently released a series of videos that go more in depth into gene therapy which can be found [HERE](#). There's also a short NBDF FAQ section [HERE](#).