

Questions About Gene Therapy in Hemophilia?



You and your patients may have questions about gene therapy in hemophilia. While more information will become available over time, the following points may help provide some initial answers or clarification.

Q. Who is eligible for gene therapy?

Treatment with gene therapy is not appropriate for all patients with hemophilia. For example, at present, pediatric patients, patients with anti-adenovirus (AAV) neutralizing antibodies (NAbs) above a certain threshold, and patients with potential liver issues or other diseases may not be eligible. At this time gene transfer research in hemophilia has only focused on patients with moderately-severe or severe hemophilia.¹⁻³

Q. What might be important to discuss with my patients?

Your role in enabling shared decision-making will remain critical to empower patients to make the best decisions for themselves. If gene therapy is approved to treat hemophilia in the future, it will be important to discuss the unique aspects of this treatment with your appropriate patients including eligibility for gene therapy, safety considerations, post-infusion follow-up requirements including long-term monitoring, and more.^{4,5}

Q. If a patient might be appropriate and eligible for gene therapy, what other requirements do they need to understand?

Patients should understand that gene therapy will come with some responsibility following the infusion, including a period of monitoring and lifestyle changes that may be required after treatment.³⁻⁶

Q. Can gene therapy be re-dosed?

As part of the humoral immune response, neutralizing antibodies may develop following single administration of gene therapy. As a result, this could impact the potential for re-administration. Research is currently ongoing to learn more about the immune response and potential strategies to overcome this for re-dosing. Most current gene therapy trials exclude patients who have already received gene therapy.^{1,7-8}

THE CHOICE TO UNDERGO TREATMENT WITH GENE THERAPY IS AN IMPORTANT ONE.

When the time comes, you and your patient can work together to determine which, if any, option is best.

References:

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