

BUSTING MYTHS

ABOUT GENE THERAPY RESEARCH IN HEMOPHILIA

Hemophilia is a rare genetic bleeding disorder that causes a delay in clot formation as a result of a deficiency in blood-clotting factor. One emerging area of research in hemophilia is gene therapy, aimed at determining whether a new or functional gene can be used to restore the function of a mutated gene.

Research in this area is still ongoing, but there may be a few misconceptions around gene therapy research in hemophilia that are important to dispel.



TRUTH OR MYTH?

Gene therapy for hemophilia would prevent a treated person from passing the disease on to their children.

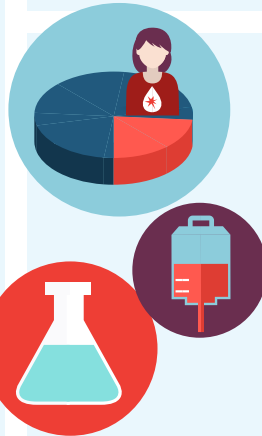
MYTH: Hemophilia is typically an inherited disease. While gene therapy research is hoping to determine whether a new or functional gene can be used to produce working clotting factor, it is not intended to change or edit the gene which does pass onto the next generation.



TRUTH OR MYTH?

You don't need a specific mutation to potentially benefit from hemophilia gene therapy.

TRUTH: Gene therapy research is aimed at addressing the defective clotting factor, not a specific genetic mutation. The goal of this approach is to deliver a corrected copy of the defective gene in order to produce factor protein.



TRUTH OR MYTH?

Hemophilia is *always* inherited, meaning it's passed through the parents' genes or DNA.

MYTH: In one-third of cases, hemophilia is caused by a new or 'spontaneous' mutation.



TRUTH OR MYTH?

Gene therapy research hopes to determine if a treatment could mean a lifetime of freedom from symptoms.

TRUTH: Ongoing observation will improve our understanding of gene therapy's potential safety, efficacy and durability after a single dose.



TRUTH OR MYTH?

Gene therapy research for hemophilia is a recent idea and not frequently studied.

MYTH: Scientists have been investigating and evolving the field of gene therapy for more than 50 years. To date, more than 2,600 gene therapy clinical trials are planned, ongoing, or have been completed for different genetic diseases. In fact, Hemophilia is one of the most extensively researched diseases in the field of gene therapy.

FOR MORE INFORMATION ABOUT GENE THERAPY RESEARCH FOR HEMOPHILIA:

Check out www.hemophiliaforward.com