

# 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 deficiency in one of several blood-clotting factors. 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, or inactivate, a mutated gene.

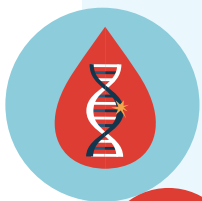
Research in this area is still ongoing, but there may be a few misconceptions around gene therapy research in hemophilia that are important to dispel.



### TRUTH OR MYTH?

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

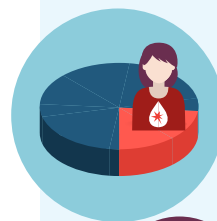
**MYTH:** Hemophilia is typically an inherited disease. While gene therapy research is hoping to determine whether a new or functional gene can be used to produce normal clotting factor, it is not intended to change or edit the gene which does actually pass onto the next generation.



### TRUTH OR MYTH?

**You don't need a specific mutation to participate in a clinical trial for hemophilia gene therapy.**

**TRUTH:** Gene therapy research is aimed at addressing the defective clotting factor, not a specific genetic mutation.



### TRUTH OR MYTH?

**Hemophilia is *always* an inherited genetic trait.**

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



### TRUTH OR MYTH?

**Gene therapy research aims to determine whether a single administration of a therapy could eliminate the symptoms of a disease.**

**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, multiple investigational gene therapies are now being researched in clinical trials!

FOR MORE INFORMATION ABOUT GENE THERAPY RESEARCH FOR HEMOPHILIA:

Check out [www.hemophiliaforward.com](http://www.hemophiliaforward.com)