

Gene Therapy Resources: Educating the Community

Future Therapies Section of the NHF Website

- Frequently Asked Questions (FAQs)
- Gene Therapy Glossary
- What Is Gene Therapy Video
- Webinar – All About Gene Therapy
- News
- And More!



JOIN US FOR

Gene Therapy: Getting Up to Speed

An in-depth webinar with Dr. Steven Pipe
Wednesday, February 19th
at 7PM EST

insights
INNOVATIONS IN TREATMENT

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FREQUENTLY ASKED QUESTIONS ON GENE THERAPY

GENE THERAPY DEFINED:
What is gene therapy?
Gene therapy is a technique for introducing genetic material into cells to compensate for abnormal genes or to make a beneficial protein. It is an experimental approach to treating a wide variety of diseases, including inherited disorders, cancer, and HIV/AIDS. The goal is to replace or supplement a defective or missing gene with a functional one.

What is a vector?
A vector is a carrier for the DNA or RNA of interest. It is used to deliver the genetic material into the target cells. Vectors can be derived from viruses, bacteria, or other organisms. They are modified to ensure they are safe and effective for gene delivery.

What are the different types of vectors?
There are several types of vectors, including viral vectors (retrovirus, lentivirus, adenovirus, herpesvirus), non-viral vectors (liposomes, nanoparticles), and exosome vectors.

What are the advantages and disadvantages of gene therapy?
Advantages: Potential for long-term or permanent treatment, reduction in the need for lifelong medication, and the ability to target specific cells. Disadvantages: High cost, potential for immune response, and the risk of insertional mutagenesis.

What is the current status of gene therapy?
Gene therapy is an active area of research. Several clinical trials are ongoing, and some gene therapies have been approved for use in the United States and Europe.

What is the future of gene therapy?
The future of gene therapy is bright. Advances in vector technology and gene editing tools like CRISPR-Cas9 are expected to expand the range of diseases that can be treated.

PUSHING THE BOUNDARIES OF TREATMENT FOR HEMOPHILIA A AND B

15

THE 15TH WORKSHOP ON NOVEL TECHNOLOGIES AND GENE TRANSFER FOR HEMOPHILIA SURVIVES THE FUTURE

The 15th Annual Workshop on Novel Technologies and Gene Transfer for Hemophilia was held in Washington, DC, on November 15-16, 2019. The workshop was organized by the National Hemophilia Foundation and the National Hemophilia Research Center. The workshop focused on the latest advances in gene therapy and gene transfer technologies for the treatment of hemophilia. The workshop was attended by experts in the field of gene therapy and gene transfer, including Dr. Steven Pipe, Dr. Michael B. Zuckerman, and Dr. Robert M. Waymouth. The workshop was a success, and it provided a valuable opportunity for researchers and clinicians to share their knowledge and experiences.

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GENE THERAPY GLOSSARY OF TERMS

A **Adeno-associated virus (AAV):** A small, non-replicating virus that is used as a vector for gene delivery. AAV is derived from the parvoviridae family and is known for its ability to infect a wide range of cells.

B **Adenovirus:** A virus that is used as a vector for gene delivery. Adenovirus is derived from the adenoviridae family and is known for its ability to infect a wide range of cells.

C **CRISPR-Cas9:** A gene editing technology that allows for precise editing of the genome. CRISPR-Cas9 is derived from a natural bacterial defense mechanism and is used to create targeted mutations in the DNA.

D **Exosome:** A small, membrane-bound vesicle that is released by cells. Exosomes are used as a non-viral vector for gene delivery.

E **Gene therapy:** A technique for introducing genetic material into cells to compensate for abnormal genes or to make a beneficial protein. Gene therapy is an experimental approach to treating a wide variety of diseases, including inherited disorders, cancer, and HIV/AIDS.

<https://www.hemophilia.org/Bleeding-Disorders/Future-Therapies/Resources>