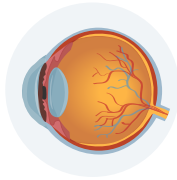


GENE THERAPY AND GENE-MODIFIED CELL THERAPY IN RARE DISEASES



Gene therapy and gene-modified cell therapies offer the potential to help patients with rare diseases to cure or improve their disease



Leber congenital amaurosis



Spinal muscular atrophy



Beta-Thalassemia



Adrenoleukodystrophy



Hemophilia A and B

There are many other rare diseases that gene therapy is currently being developed for

1. Patient Education

Patients should be **informed and educated** on gene therapy

2. Shared-Decision Making and Informed Consent

Implementation of gene therapy requires a **multidisciplinary approach** with the patient being central



3. Follow-up

Patients should be included in a **registry** to ensure **long-term follow-up**

FURTHER READING



[Gene Therapy Basics \(2022 Update\)](#)¹

[Gene Therapy: The Basics – FAQs](#)²



[Landscape Report](#)³

[American Society of Gene + Cell therapy](#)

[Educational Resources Directory](#)⁴

[European Consortium for Communicating Gene & Cell Therapy Information](#)

[E-learning gene therapy and hemophilia](#)⁵

[World Federation of Hemophilia](#)



[Papaioannou, et al.](#)⁶

Clinical applications of gene therapy for rare diseases: A review

2023

[Samelson-Jones, et al.](#)⁷

Adeno-Associated Virus Gene Therapy for Hemophilia

2023

[Hermans, et al.](#)⁸

How to translate and implement the current science of gene therapy into haemophilia care?

2023

Gene therapy is expected to become a **major treatment approach** for many rare diseases in the future

1. Gene Therapy Basics (2022 Update), American Society of Gene + Cell Therapy, ASGCT, <https://www.youtube.com/watch?v=kAtd9X29SdQ>; 2. Gene Therapy: The Basics – FAQs, National Bleeding Disorders Foundation, <https://youtu.be/USUYAKkCrB0?si=6tidK25jOr6ZaAi>; 3. Gene, Cell, & RNA Therapy Landscape Report, American Society of Gene + Cell Therapy, ASGCT, <https://asgct.org/publications/landscape-report>; 4. Educational Resources Directory, EuroGCT, <https://www.eurogct.org/discover-gene-and-cell-therapy/educational-resources-directory>; 5. eLearning WFH educational platform, World Federation of Hemophilia, <https://elearning.wfh.org/resource/gene-therapy-for-hemophilia-what-why-how-when-who-and-where/>; 6. Papaioannou I, et al. Int J Exp Pathol. 2023;104:154-176; 7. Samelson-Jones BJ and George LA. Annu Rev Med. 2023;74:231-247; 8. Hermans C, et al. Ther Adv Hematol. 2023;14:20406207221145627

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