Gene Therapy and Biomedical Applications

Sarah Dundore | Dr. Pattarkine
BTEC 250 SP 2022

Abstract

Gene therapy has many uses today through the new treatment options and it's potential to treat uncurable diseases

Introduction

- Utilizing genes, DNA or RNA components, to treat conditions rather than pharmaceutical drugs.
- Helps with synthesis of functional proteins which could be missing, faulty, or mutated due to the condition
- Two types of gene therapy: somatic and germline
- Two modes of delivery: *in vivo* and *ex vivo*

Novelty of Work

- Less side effects, less chances of rejection
- Allows treatments for non-treatable diseases

Methodology

Vectors are modes to transfer the genes inside targeted cells and are used to protect and incase the gene
Types of Vectors:
- Plasmid – circular DNA molecules
- Viral – modified viruses

Vectors containing corrected DNA/RNA can be inserted directly into the patient or *ex vivo*
One way vector can be inserted is through intravenous
Patient cells can be removed and exposed to vector before intravenous insertion

Examples

Table 1: FDA/EMA approved gene therapy products [5]

Name	Year	Agency	Indication
Yescarta	2017	FDA	Lymphoma
Kymiah	2017	FDA	Leukemia
Strimvelis	2016	EMA	ADA-SCID

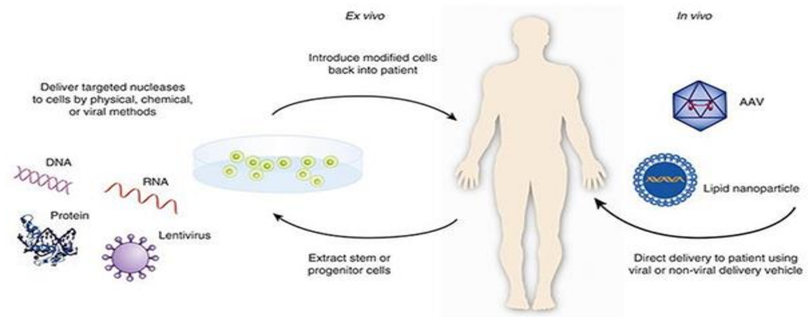

Figure 1: Demonstration of ex vivo and in vivo approaches [2]

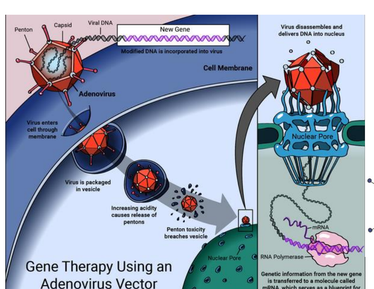

Figure 2: Mode of action for gene therapy [3]

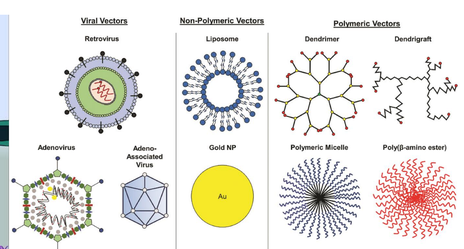

Figure 3: Types of vectors used in gene therapy

Discussion

Gene therapy is heavily tested to make sure the safety and effectiveness of the therapy
Currently many gene therapies released and approved by FDA, or similar, approved
Some challenges are primarily due to the types of viral vectors leading to cancer or nonspecific binding.
The future of gene therapy reaches into possible repairing of damaged tissues and alternative cancer treatments

Conclusion

Despite the drawbacks, gene therapy could be the answer to many genetic disorders being treated or even eradicated
With obvious challenges, gene therapy could be the only option for treating several genetic and chronic conditions (Cancer)

References

[1] Caffery, et alNanomaterials 2019, 9, 105. \
[2]Center for Biologics Evaluation and Research. (2018). U.S. Food and Drug Administration.
[3] MedlinePlus Genetics. (2022). MedlinePlus.
[4] Office of the Commissioner. (2017). U.S. Food and Drug Administration.
[5] Ginn, et al. (2018). The Journal of Gene Medicine. 20. e3015. 10.1002/jgm.3015.