Gene Therapy: Implications for Pharmacy Practice

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Disclosures

- I have not been bribed
- I have not been corrupted
- I have no hidden alliances with rich people
- My motives are pure

Learning Objectives

- Define the foundation and general principles of gene therapy
- Review practices and handling of gene therapy products
- Describe issues that pharmacists should address now and in the future

Gene therapy involves the introduction of genetic material into an individual, or the modification of the individual's genetic material, in order to achieve a therapeutic objective

The World Health Organization

Therapeutic options

- Single genetic conditions
- Multifactorial genetic conditions
- Acquired genetic conditions

Vectors

- Viral
- Liposomal
- Artificial Chromosomal
- Other nucleic acid (plasmid, RNA technology)

Viral vectors

- Retrovirus
- Adenovirus
- Adeno-associated virus

Vector	Advantages	Disadvantages
Adenovirus	High transfection efficiency in vivo and ex vivo Can infect dividing and nondividing cells Wide host cell range	Limited insert size capacity Short duration of expression Immunogenic (repeat dosing therefore ineffective)
Retrovirus	No immune response Reasonable duration of expression Integrates into host cell genome High transfection efficiency ex vivo	Only infects dividing cells Limited insert size capacity Potential safety risk of insertional mutagenesis
Adeno-associated virus	Can infect dividing and nondividing cells Reasonable duration of expression Low immunogenicity	Inefficient large scale virus production Very limited insert size capacity

Technical Limitations

- Identification
- Manufacturing
- Introduction
- Expression

Patient Safety

- Toxicity
- Fatality
- Interactions
- Latent effects

Ethics

- Selection
- Repair versus improvement
- Consequences

Follow-up

Biosafety Levels

- Biosafety Level 1
- Biosafety Level 2
- Biosafety Level 3
- Biosafety Level 4

Biosafety Level 2

- Standard Microbiological Practices
- Special Practices
- Safety Equipment
- Laboratory Facilities (Secondary Barriers)

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Pharmacy Handling

- Receipt and storage
- Preparation
- Dispensing
- Disposal
- Decontamination of spills
- Accidental exposure

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Conclusion

- Increased availability leads to increase in knowledge
- Increased knowledge leads to decreased uncertainty.
- Decreased uncertainty leads to confidence to select the appropriate level of caution
- Advisory Group on Gene Therapy

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