Gene Therapy Evolution: How Gene Therapy has Evolved Preventive Medicine

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Gene Therapy Evolution
How Gene Therapy has Evolved Preventive Medicine
Kayuri Shah, Virginia Commonwealth University

Introduction
Gene therapy is defined as a regenerative therapy that contains an active substance containing recombinant nucleic acid administered to human beings with the intention of regulating, repairing, replacing, adding, or deleting a genetic sequence. The goal of gene therapy is to have a therapeutic or diagnostic effect that relates directly to the desired gene expression. The techniques required for gene therapy can help several areas that have gone untreated, such as skeletal diseases, brain tumors, and hereditary diseases. The main focus over the years has been an on bone regeneration avenues through gene therapy. Though gene therapy has suffered several setbacks during clinical trials due to errors within human trials, with the support of public research institutions, the media, and the public, research has picked up the pace. However, how much has gene therapy helped in the advancement of preventive medicine and bone regeneration? Gene therapy researchers have yet to define gene therapies’ areas of applications. Applications range from ex vivo and in vivo, directly altering the gene in vivo. The ethical and safety concerns are vast if the procedure is used improperly, and cannot proceed before a line is defined between actual physical hindrances and cosmic enhancement, meaning the difference between creating dwarfism and an undesirable height. Without the ability to perform the proper clinical trials, researchers argue there will be no furthering of gene therapy to actual human application on a wide market, available to everyone. Despite the ethical and safety concerns, in order to advance preventive medicine in bone regeneration, gene therapy protocols need to reach the clinical level, but new regulations will not allow that before testing is completed on larger animals. Since there are several methods, such as ex vivo and in vivo, each able to use viral and non-viral vectors, preventive medicine can improve through the use of gene therapy.

Various Gene Therapy Methods
- **In vivo viral vectors**
  - Vector technology: high efficiency by viruses transferring genetic information into host cell
  - Overexpression of genes lead to faster healing
  - Tested in rat models
- **In vivo non-viral vectors**
  - Less costly
  - Safer
  - Minimal immune response from host

- **Ex vivo viral vectors (traditional, cell-mediated)**
  - Outside host’s body: foreign DNA is manipulated with target cells outside the body
  - Tested in large animals
  - Can be used in combination with several types of vectors

- **Ex vivo non-viral vectors (expedited, cell-mediated)**
  - Technically combining ex vivo and in vivo methods
  - Least experimented with, yet most likely to be used in an operating room

Evolution of Gene Therapy

![Timeline of Therapeutic Events](image)

Timeline of Therapeutic Events

<table>
<thead>
<tr>
<th>Year</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>1989</td>
<td>First use of retroviral vectors in gene therapy.</td>
</tr>
<tr>
<td>1990</td>
<td>First clinical trial of gene therapy for cancer.</td>
</tr>
<tr>
<td>2000</td>
<td>First clinical trial of gene therapy for HIV/AIDS.</td>
</tr>
<tr>
<td>2010</td>
<td>First clinical trial of gene therapy for cardiovascular disease.</td>
</tr>
</tbody>
</table>

Future Goals
1. What regulations will be required to ensure the safe usage of gene therapy?
2. Before clinical trials can be reached, what are the next step for researchers to take so the methods can start being applied to humans again, after several setbacks?
3. What factors determine the best use of the therapeutic methods?
4. How far along is the rest of the world in this research?

Conclusion
Preventive medicine has reached a long way through gene therapy, definitely helping the science behind bone formation. However, the largest obstacle that is still presented is the safety concerns surrounding each method, and without testing on larger animal models, there is no way to know how the therapy products would react on a human. The pitfall of regenerative medicine is that it can only be done on live models, and therefore, must also be carefully monitored in its usage. Regardless of the setbacks in gene therapy, the regulations that have been set in place by the US FDA, suggested by the NIH and other researchers, have allowed for safer clinical trials. Not only do we want to prevent deaths, but the misuse of the methodology. Gene therapy has yet to be completely defined in its purpose; no line has been drawn between using the products for cosmic reasons, or using it for actual genetic conditions. Neither has anyone explored the possibilities of what may happen if gene therapy is done in utero, and the implications on the mother, baby, and society. Current legislation only allows gene therapy to be conducted in somatic cells, but that does not mean there will not be those that attempt to perform it in gamete cells as well. However, in many other countries, gene therapy products are already on the market, such as Europe and China, and therefore, in the United States, it is necessary that we progress to the next level, in order to bring this medicine to the public in a safe, yet quick and efficient manner.