The basis of gene therapy:
from the molecule to the treatment

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Introduction and Aims
Gene therapy raised recently as one of the most promising techniques in the field of medicine. Although it is still in the preclinical phase in most diseases up-to-date success stories attest the enormous possibilities. However, the majority of the population barely knows about the potential of gene therapy, even those that have relatives suffering from diseases that can be treated through this technique. The aim of this project is to provide a comprehensive guide of the main aspects of the actuality of gene therapy regarding to the most recent literature. Questions of variable scope are treated, including sociological, ethical and biological issues. Finally, an analysis of current situation of treatment of some genetic diseases of blood is presented to provide an example of current situation of research in different areas.

Methods
The information was gathered through a critical revision of the current literature. The informative support for the project was Office Power Point, because it visually enhance understanding of the concepts.

1. Biological Background
Some of the most important terms to understand gene therapy:
Cells > DNA > Mutations > Genetic diseases

2. Evolution of the field
SCID was the first disease to present full or partial correction of a defective gene.
2 individuals died related to gene therapy protocols.
First protocol for gene therapy approved last year.

3. Functioning of gene therapy
Necessary steps to carry out a gene therapy protocol:
Cloning DNA > Vector insertion > Target cells insertion

4. Ethical and social issues
Human trials only for therapeutic purposes.
Germ-line modifications are forbidden by the NIH.
Genetic enhancement may become available for rich.

5. Blood diseases
SCID: First disease to show palliative effects by gene therapy.
Hemophilia: First clinical trials in hemophiliacs B show great success.
Thalassemia: Successful preclinical trials support initiation of studies in human.
LPL deficiency: The clinical protocol has been released already.
Leukemia: Modified T-cells attack cancer cells; this approach shows promising potential to treat other cancers.

Conclusions
This work provides a global perspective of the field of gene therapy and its main developments thus far.

References