Gene therapy is a medical field that focuses on the genetic modification of cells to produce a therapeutic effect or the treatment of disease by repairing or reconstructing defective genetic material. The first attempt at modifying human DNA was performed in 1980, by Martin Cline, but the first successful nuclear gene transfer in humans, approved by the National Institutes of Health, was performed in May 1989. The first therapeutic use of gene transfer as well as the first direct insertion of human DNA into the nuclear genome was performed by French Anderson in a trial starting in September 1990. It is thought to be able to cure many genetic disorders or treat them over time.

The concept of gene therapy is to fix a genetic problem at its source. If, for instance, a mutation in a certain gene causes the production of a dysfunctional protein resulting (usually recessively) in an inherited disease, gene therapy could be used to deliver a copy of this gene that does not contain the deleterious mutation and thereby produces a functional protein. This strategy is referred to as gene replacement therapy and is employed to treat inherited retinal diseases.

In the present book, thirteen typical literatures about gene therapy published in international authoritative journals were selected to introduce the worldwide newest progress, which contains reviews or original research on gene therapy. We hope this book can demonstrate advances in gene therapy as well as give references to the researchers, students, and other related people. [1]

[1] https://en.wikipedia.org/wiki/Gene_therapy