Gene Therapeutics: Past, Present and Future

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Human Gene Therapy has certainly had its ups and downs during the last two decades. In recent years, with signs of clinical improvement in diseases that include hemophilia B, inherited blindness, leukemia, immunodeficiencies and hemoglobinopathies, there has been a resurgence in the excitement in the field that was once spurned by scientists, physicians, and the biopharmaceutical industry. I will provide a historical perspective on the development of gene therapy. To do this, I will focus on but not exclusively restrict my discussion to rAAV-based hemophilia gene therapy. The preclinical studies to support the early clinical trials, the surprises that occurred in these first trials, and how these influenced the ongoing trials will be reviewed. I will also discuss some of the remaining scientific and technical hurdles that are required before a one-time curative therapeutic can be achieved. Finally, I will describe newer technologies on the horizon that might supplant current gene therapy approaches.