Special Issue

Gene Therapy Technology: Advances, Challenges and Perspectives

Message from the Guest Editors

Gene therapy has been considered the holy grail for the treatment of inherited diseases. This approach became possible through the advances of genetics and bio-engineering that enabled the manipulation of viral vectors for the delivery of extra-chromosomal material to target cells. The viruses are more often investigated due to their efficiency of invading cells and inserting their genetic material. However, there is great concern regarding exacerbated immune responses toward the injected vectors and genome manipulation, especially in germ line cells.Moreover, some limitations (i.e neutralizing antibody, tissue tropism, loading capacity) of viruses boosted the research in the development of alternative non-viral delivery strategies that are currently under investigation, but far for being optimized. Overall, despite in vivo studies showed satisfactory results with approved protocols in clinical trials for various inherited diseases, gene therapy technology still needs supporting experimental evidences, with lights and shadows that need further investigation/optimization.
Keywords:Gene therapy; viral vectors; gene delivery; genome/base editing; cell therapy

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