The use of CRISPR/Cas in clinical trials.

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Abstract

ATMPs (Advanced Therapy Medicinal Products) comprises cell therapies, gene therapeutics and tissue engineered products. Gene therapies include treatment with small oligonucleotides, gene-modified cells and DNA or viruses containing genes.
Clustered regularly interspaced short palindromic repeats (CRISPR)/CRISPR associated nuclease 9 (Cas9), as a powerful genome-editing tool, has revolutionized genetic engineering. CRISPR/Cas9 has been used for the identification of potential molecular targets for cancer therapy. In addition, CRISPR/Cas9 and other gene editing techniques are being developed for treatment of patients with AIDS, cancer and other diseases.
Gene editing is not only a promising new branch treatment for severe diseases but may also be used in sports medicine. This raises the issue whether these techniques might be abused in the field of elite sports. Both the World Anti-Doping Agency (WADA) and the International Olympic Committee (IOC) have expressed concerns about this possibility. As a result, the method of gene doping has been included in the list of prohibited classes of substances and prohibited methods.
Current detection methods are insufficient to detect gene doping because they are limited to the detection of foreign drugs or proteins and unable to measure small changes in native human protein levels. New detection methods based on sequencing are the most promising preventive methods to counteract the possible application of gene doping.