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Artificial chromosomes for gene therapy

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Artificial chromosomes (ACs) are a promising gene therapy tool and have many advantages over current vector systems which mainly involve retroviruses. Artificial chromosomes are micro-chromosomes that are developed by researchers, and have the ability to carry new genes that can perform a variety of functions and behave as endogenous chromosomes without the need for integration of the transgene into the host genome. Artificial chromosomes have the ability to overcome the limitations in transgene expression efficiency. ACs do not pose the threat of creating mutations in the DNA, activating oncogenes or leading to adverse immune responses. Different categories of artificial chromosomes are being developed namely human artificial chromosomes (HACs), yeast artificial chromosomes (YACs), bacterial artificial chromosomes (BACs) and P1-derived artificial chromosomes (PACs). Among these, human artificial chromosomes (HACs) are a better option for gene therapy protocols because the other ACs can disrupt the original human genome as they have different expression levels when compared to HACs. HACs are also more stable and safe. Another application of HACs is that they can be used in animal models to produce therapeutic products and for research on various human diseases. A major challenge in developing artificial chromosomes is the complication of synthesising and characterising centromeres located on repetitive α-satellite DNA which are defined epigenetically. There is ongoing research to understand the epigenetic machinery to trigger the identity of centromeres without α-satellite DNA to build a new generation of HACs. With further progress in research, artificial chromosomes could increase the therapeutic potential of gene therapy and can be used in the treatment of inherited diseases, such as haemophilia or Friedreich’s ataxia.

Keywords: Artificial chromosomes, Gene therapy, Transgene, Human artificial chromosomes, Centromeres, ÃŽÂ±-satellite DNA

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