Novel, Virus-Independent In-Vivo Gene Therapy Approach

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Technology description

Description

A novel, highly efficient method has been developed that allows therapeutic gene delivery directly into tissues such as skin, tendons, ligaments, or muscles. The approach is applicable to the treatment of a wide range of diseases and traumas such as cancer, rheumatoid- and osteo-arthritis, osteoporosis, muscular dystrophies, and tendon or ligament damage. Gene delivery does not require the use of viral vectors, thereby eliminating the danger of anti-viral immune responses, and has been shown to be highly efficient both ex vivo and in vivo in rabbit and dog models of osteochondral defect repair and flexor tendon healing.

Using this method, greater than 70 percent of primary rabbit perichondrium and cartilage cells transfectedin vitrowere positive for the introduced gene. After reintroduction into the rabbit knee, the cells continued to express the transgene for at least a week. The transfection method also allows for introduction of the therapeutic gene by direct injection into the appropriate site. With this type of delivery the gene becomes distributed approximately one hundred cell layers deep in the tissue, in contrast to viral delivery, which generally distributes the gene only a few cell layers deep. In addition to being highly efficient, it is relatively quick to perform adding only a few extra minutes to the total time of surgery.

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