Gene therapy for cartilage repair: current status and future perspectives

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Articular cartilage defects resulting from acute trauma and osteoarthritis have a limited capacity for regeneration. As none of the current clinical options are capable of reproducing the natural structure and functions of the native tissue, active work is ongoing to define novel, effective procedures to treat cartilage lesions. Gene therapy may offer strong tools to enhance cartilage repair by delivering genetic candidate sequences in the repair tissue for the sustained production of therapeutic agents in sites of injury. Recently, gene therapy approaches have been combined with the use of tissue engineering systems for the improved, controlled delivery of gene transfer vectors via biocompatible materials to elaborate optimal treatments for cartilage lesions. An overview is provided here on the most recent concepts of gene therapy for cartilage repair, with a focus on recombinant adeno-associated virus (rAAV) vectors as adapted gene vehicle for human gene therapy.

Dr. Cucchiarini is currently an Associate Professor in Molecular Biology at the Saarland University and Saarland University Medical Center, Homburg/Saar, Germany (currently working at the Center of Experimental Orthopaedics). Before joining the Saarland University, she worked as a senior post-doctoral fellow at the Harvard Institutes of Medicine, Boston, MA, USA in the field of anti-HIV CNS gene therapy. Prior to moving to the USA, she worked at the University Hospital Inselsspital, Bern Switzerland on HCV immunology and vaccination at the Department of Internal Medicine. Dr. Cucchiarini teaches in the fields of Biochemistry, Molecular Biology, and musculoskeletal gene therapy and tissue engineering. She completed her Ph.D. in 1995 at the University of Nice-Sophia Antipolis, Nice, France in Life Sciences, working on HIV peripheral gene therapy, providing teaching sessions in Virology and Immunology.