Use of Gene Therapy to Treat Joint Disease and Synovial Tumors

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BACKGROUND

The National Center for Advancing Translational Sciences and Genetic and Rare Diseases Information Center characterizes Pigmented villonodular synovitis (PVNS) as a rare disease estimated to occur in ~ 5-6 people out of 100,000. This locally invasive tumor most often occurs in younger adults and causes severe damage to joints. The first line of treatment is surgery but at least 50% of patients require multiple surgeries over many years due to re-growth of the tumor.

TECHNOLOGY DESCRIPTION

Researchers at UC San Diego discovered unique gene delivery vectors that markedly increase the efficiency of transduction in synovial tissue and synovial cells. These vectors can be used to deliver a variety of transgenes to treat synovial tumors, most notably pigmented villonodular synovitis (PVNS). In addition, these vectors can be used to deliver a variety of transgenes to treat various joint diseases, whereby apoptosis regulating genes that can kill synovial cells resulting in a functional and anatomical improvement in a treated joint.

APPLICATIONS

The transgene is injected into the patient's diseased joint or tumor in conjunction with the administration of the proper vectors. The therapy will avoid repeated surgeries, which can lead to disability and pathologic fractures.

ADVANTAGES

Current vectors are relatively inefficient and high concentrations are needed to infect synovial cells. The newly engineered vector increases efficiency dramatically. In addition, synovial cells are relatively resistant to apoptosis. We discovered that this can be overcome with specific transgenes that bypass this resistance, most notably PUMA and other members of the BH3-only Bcl2 family members. For PVNS, patients could avoid repeated surgeries and could instead be treated with intra-articular therapy.

STATE OF DEVELOPMENT

In vitro proof of concept using cultured synovial cells has been completed and confirms the ability of the construct to efficiently kill the synovial cells. In vivo efficacy has been demonstrated in inflammatory joint disease in pre-clinical models.

INTELLECTUAL PROPERTY INFO

This invention is covered by a recently granted patent.

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20160243194	08/25/2016	2012-414

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OTHER INFORMATION

KEYWORDS

Apoptosis, Rheumatoid arthritis, autoimmune disease, arthritic joint, synovial cells, gene therapy, recombinant adenovirus

CATEGORIZED AS

- **Medical**
  - Delivery Systems
  - Disease: Autoimmune and Inflammation
  - Disease: Cancer
  - Gene Therapy

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