Technology
Methods and compositions of novel gene therapy designed to stimulate bone synthesis for treatment of osteoporosis or bone fractures

Inventor
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Key Features
- Gene therapy that can be delivered either locally or systemically
- Results in a marked increase in bone synthesis and healing
- Promotes angiogenesis

Stage of Development
Reduced to practice with successful demonstration of methodology in animal models

Keywords
Therapeutic
- Osteoporosis
- Bone regeneration
- Gene therapy

Patent Status
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Ex vivo and in vivo Genetic Therapies for Bone Regeneration
(VA Reference No. 07-017)

Methods and compositions of novel gene therapy designed to stimulate bone synthesis for treatment of osteoporosis or bone fractures

Technology
The Department of Veterans Affairs has developed gene therapy-based technologies designed to stimulate bone synthesis, promote stem cell renewal, and prolong therapeutic efficacy in the treatment of osteoporosis or in the rapid healing of bone fractures.

Description
The novel method developed entails importing recombinant DNA specifically into osteoblast nuclei or osteoblast stem cells that can be stably transduced with a gene encoding a potent bone-promoting growth factor. The modified stem cells can be injected into a patient for systemic action. As an alternative to systemic treatment with modified stem cells, osteogenic growth factors can be localized to the site of a bone fracture or weakening through retrovirus or lentivirus-based gene therapy. Therapy for this particular method would be in the form of a resorbable scaffold or a solution that would be injected into the fracture site. Following gene transfer, transduced cells express the modified transgenic osteogenic factor, resulting in high-sustained levels at the fracture site and markedly increasing bone synthesis and healing.

Competitive Advantage
Ideal therapies for weak or broken bones would be minimally invasive and of sufficient duration to promote osteogenesis, damaged bone resorption, and angiogenesis (to provide newly deposited tissue with nutrients).

This therapy:
- Can be delivered locally or systemically, depending on the pathological conditions.
- Can provide extended therapy for chronic osteoporosis because the stem cells used in ex vivo gene therapy regenerate.
- Allows targeting to the proliferating periosteal cells that arise shortly after a fracture event resulting in high-sustained levels of the osteogenic growth factor while also promoting angiogenesis.

Status
The Department of Veterans Affairs is looking for a partner for further development and commercialization of this technology through a license and the VA inventors are available to collaborate with interested companies through a Cooperative Research and Development Agreement (CRADA).