Treatment for Osteogenesis Imperfecta

**OCR Number:** OCR 6436

**Description:**

**PEDF and Derivative Peptide for Treatment of Osteogenesis Imperfecta**

- Absence of pigment epithelium-derived factor (PEDF) causes Osteogenesis Imperfecta (OI) in humans.
- OI Type VI is an autosomal recessive disease manifested by severely impaired bone mineralization and fractures in early childhood.
- PEDF is a regulator of MSC differentiation to the osteoblast lineage. PEDF modulates Wnt/β-catenin signaling to direct MSC fate toward osteoblasts. Restoration of PEDF in this PEDF KO mice corrected the bone phenotype (figure).
- Recently it was shown that PEDF treatment restores bone elasticity and reduces bone brittleness in the PEDF-KO mouse model (Unpublished data).
- **Reference:** Gattu *et al.* "Determinant of mesenchymal stem cell fate by pigment epithelium-derived factor (PEDF) results in increased adiposity and reduced bone mineral content." The FASEB Journal 27.11 (2013): 4384-4394.

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