A Small Molecular Compound for Arthritis and Bone Erosion Treatment

Technology #7656

**Unmet Medical Need:** Bone damage is a devastating disease outcome for many millions of patients with arthritis or other bone-destroying conditions. Despite the advent of biologic agents, treating erosive bone damage remains a challenging endeavor. Currently used biologic agents and other modern drugs are very expensive, have significant side effects, and most of them can only be administered parenterally. The proposed solution offers a potent and specific treatment modality. To the best of our knowledge, it is the first to address both immune and osteoclast (OC) dysregulation with a unified therapeutic approach.

The solution proposed here has several innovative and unique advantages over existing drugs:

- A novel target and a well-mapped binding site;
- A well-characterized lead: [Small size (MW 330 Da); pM-range *in vitro* effects; modestly orally available, but with strong effect *in vivo* at mg/Kg-range dosing, twice weekly by gavage]
- The target-mediated pathway has been validated *in vitro* in both human and mouse cell systems, as well as in mouse pre-clinical models.
- Dual effect on inflammation and bone damage
- Different from established or emerging treatment modalities which all target nonspecific, ‘downstream’ mechanisms, the approach proposed here targets an ‘upstream’ highly specific molecular interaction event. As such the proposed solution is unlikely to produce side effects.
- Given the direct effect of the compounds on OC, they might be useful drugs in non-arthritic OC-mediated bone erosive conditions as well.

**Inventors**

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