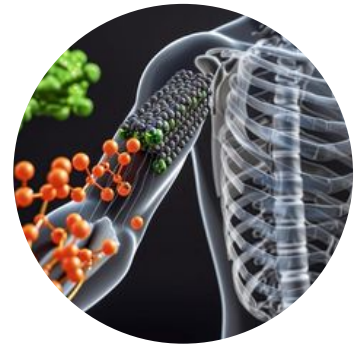


Novel Therapeutic Approaches for Genetic Skeletal Diseases and Targeted Delivery to Bone

A leading company in rare disease therapeutics is seeking **novel treatments for rare genetic skeletal diseases** and genetically defined subsets of common skeletal diseases. They are also interested in **delivery techniques to target bone tissues**, ideally coupled with novel therapeutics.



Approaches of Interest:

- **Priority modalities of interest:** non-viral therapeutic approaches, mRNA, oligonucleotides (e.g. RNAi, siRNA, ASO, and saRNA), small molecules, protein replacement and substitution, among other novel approaches.
- **Novel approaches to target delivery to bone** are also of interest, including peptides, lipids, glycans, antibodies, aptamers, modified oligonucleotides, nanoparticles, extracellular vesicles, circulating factors, small molecules or unique delivery devices and routes of administration.

Combination of a novel therapeutics approach with a bone-targeting mechanism will be reviewed as a high priority, but separate approaches in either area of interest.

Clinical Indications of Interest: Apert Syndrome, Autosomal Dominant Osteopetrosis, Ehlers-Danlos Syndrome, Hereditary Multiple Exostoses/Osteochondromas, Hypophosphatasia, Osteogenesis Imperfecta, Pseudoachondroplasia, Spondyloepiphyseal Dysplasia Congenita, Stickler Syndrome, and X-Linked Hypophosphatemic Rickets. Genetic subsets of more common disease (E.g. osteoporosis, osteoarthritis).

Out of Scope:

- Approaches for skeletal oncology, infectious disease, or immunology
- Drug discovery platforms and cell therapy approaches are not of interest

Developmental Stages of Interest:

- Opportunities from preclinical research and onwards are of interest for this campaign.
- Opportunities submitted with *in vivo* validation are of highest interest, with preference for therapeutic approaches which are nearing IND ready. However, opportunities with strong *in vitro* proof of concept data also still be considered.

Submission Information & Opportunity for Collaboration

Submission of 200–300-word briefs is encouraged, along with any supplementary information e.g. relevant publications, patents or slide decks. The team encourages including the proposed next steps in developing the research towards commercialization. In submitting to this campaign, you confirm that your submission contains only non-confidential information.

Our client is looking to establish long-standing partnerships in the field of rare skeletal disease. The most appropriate outcomes for submissions to this campaign will be decided on a case-by-case basis. Example outcomes include funded research collaborations and agreements or licencing of assets.

Opportunities sought

-  Technologies
-  Academics and expertise
-  Centres of excellence
-  Research projects
-  Spinout companies
-  Biotech assets

Submissions

Please submit relevant, non-confidential opportunities online [here](#)

Deadline: **28th April 2025 - 10:59 pm GMT**

Have any questions?

Contact our team at discover@in-part.co.uk