

## Seeking Transformative Solutions to Treat Rare Skeletal Diseases

BioMarin Pharmaceutical Inc. is a world leader in developing and commercialising **first- or best-in-class therapies for rare genetic diseases**. BioMarin Pharmaceutical Inc. is looking for **expertise in the pathology and epidemiology** (highlighting a focus on clear genetic correlations) **of rare skeletal diseases**, alongside **novel approaches for therapeutic intervention and technologies for targeted delivery to various cell types of the bone**.



### Approaches of Interest

- **Therapeutic approaches to treat rare skeletal diseases**, particularly but not limited to: osteogenesis imperfecta, Ehlers-Danlos syndrome, pseudoachondroplasia, osteopetrosis, Stickler syndrome, Spondyloepiphyseal dysplasia congenita, and multiple hereditary exostosis
- **Unique and innovative therapeutic modalities are encouraged**
- **Open to different modalities** including gene correction and editing, cell therapy, viral and non-viral gene therapy, RNA interference, modified oligonucleotides, small molecules, protein replacement and substitution
- **Novel approaches for the targeted delivery to cell types of the bone**, including peptides, lipids, glycans, antibodies, aptamers, modified oligonucleotides, nanoparticles, extracellular vesicles, circulating factors, small molecules or delivery devices and routes of administration
- **Thorough transcriptomic and/or proteomic data in various cell types of the bone** in normal or diseased pathology

### Out of Scope

- Approaches targeting oncology, infectious diseases or genetic skeletal *muscle* disorders are excluded

### Developmental Stages of Interest

- Opportunities at basic research phase to Phase I clinical trial are within scope
- Opportunities with *in vivo* validation are of highest interest, although opportunities with compelling *in vitro* or *in silico* validation will still be considered





### Submission Information

Submission of one page, 200-300 word briefs are encouraged, along with any optional supplementary information e.g., relevant publications and patents. In submitting to this campaign, you confirm that your submission contains only non-confidential information.

### Opportunity for Collaboration

BioMarin Pharmaceutical Inc. is open to a range of collaboration opportunities, with the most appropriate outcome being decided on a case-by-case basis. Example outcomes include licensing assets, project/PhD funding, and research collaborations. Our client is looking to establish long-standing partnerships in the field of rare skeletal disease.

## Opportunities sought

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

## Submissions

Please submit relevant, non-confidential opportunities online via: [discover.in-part.com](https://discover.in-part.com)

Deadline: **11th April 2022 - 10:59 pm GMT**

### Have any questions?

Contact our team at [discover@in-part.co.uk](mailto:discover@in-part.co.uk)



