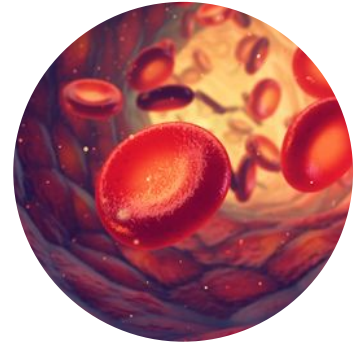


## Seeking Translational Early Discovery Research in Haematology

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 early discovery research that **involves targets with strong genetic evidence in haematology**, with a focus on haemoglobinopathies (**sickle cell disease, beta-thalassemia, and alpha-thalassemia**), coagulopathies (**haemophilia A, haemophilia B, von Willebrand disease, hereditary antithrombin-III deficiency**), or myeloproliferative neoplasms.



### Approaches of Interest

- Therapeutic approaches to treat **haemoglobinopathies**. **Of special interest are therapeutic approaches targeting different beta-haemoglobinopathies** (sickle cell disease and beta-thalassemia) with the same modality
- Therapeutic approaches to treat **coagulopathies**
- Therapeutic approaches to treat **myeloproliferative neoplasms**
- Unique and innovative therapeutic modalities are encouraged
- Open to different modalities including small molecules, biologics, oligonucleotides, and gene therapy including gene editing
- Novel approaches for *in vivo* or *ex vivo* **targeted delivery to haematopoietic stem cells, hepatocytes, or endothelial cells**, including lentivirus, adeno-associated virus (AAV) capsids, and lipid nanoparticles (LNP)

### Out of Scope

- Research focusing exclusively on ameliorating symptoms, such as veno-occlusive disease, is not supported

### Developmental Stages of Interest

- Opportunities at basic research phase to late stage clinical trial are within scope
- Opportunities with *in vivo* validation in exclusively mammalian models are of highest interest, although opportunities with *in vitro* validation will still be considered, particularly in patient-derived diseased cells






### 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. BioMarin Pharmaceutical Inc. is looking to establish long-standing partnerships in the field of haematology.

## 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: **21st February 2022 - 11:59 pm GMT**

### Have any questions?

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

