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

Deadline: 21st February 2022 - 11:59 pm GMT

Have any questions?
Contact our team at discover@in-part.co.uk

BioMarin Pharmaceutical Inc. takes pride in going where the science leads them, pioneering breakthrough treatments for debilitating and life-threatening conditions where they can significantly improve upon the current standard of care.