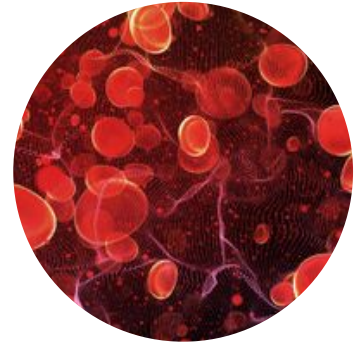


Novel Therapeutics and Druggable Targets for Non-malignant Hematological Diseases

An American pharmaceutical company with a focus on rare diseases is seeking **novel therapeutics for non-malignant hematological diseases**. Drug molecules that have a **defined target, mechanism of action, and proven efficacy** for a number of rare blood diseases are in scope for this campaign.



Approaches of Interest:

- Diseases of interest include, but are not limited to, hereditary hemorrhagic telangiectasia, sickle cell disease, thalassemia, hereditary hemochromatosis, hereditary spherocytosis, diamond Blackfan anemia, Fanconi's anemia, erythropoietic protoporphyria, warm autoimmune hemolytic anemia, and thrombocytopenia
- Modalities of interest include **small molecules, RNA-based therapeutics, proteins/peptides, and antibodies**
- Therapeutics that act on the **SPHK1, HRI, BCL11A and ALAS2** targets are of particular interest

Out of Scope:

- Ultra rare diseases and indications that require acute care
- Cell and gene therapies
- Treatments that act on the following targets: LRF/ZBTB7A, EED complex, LSD1, HIF-PHIs, FcRNs
- Diagnostic and screening methods for diseases of interest
- Repurposed or reformulated drugs and novel delivery methods
- *Ex vivo* treatments

Developmental Stages of Interest:

- Opportunities from preclinical to registration will be considered, with **late preclinical to Phase 2** opportunities being of highest interest
- Opportunities should have a minimum of **in vivo validation** for consideration





Submission Information

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

Opportunity for Collaboration

Our client 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 and research collaborations.

Opportunities sought

-  Technologies
-  Research projects
-  Spinout companies
-  Biotech assets

Submissions

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

Deadline: **27th May 2024 - 10:59 pm GMT**

Have any questions?

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