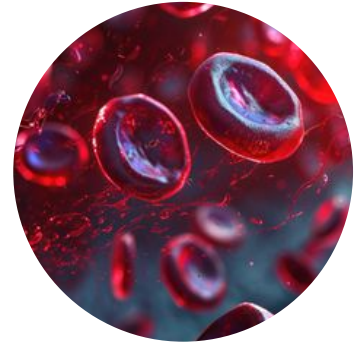


Seeking Transformational Therapeutic Approaches for Sickle Cell Disease and Beta-thalassemia

Our client, a global leader in the development of pioneering therapies for rare genetic diseases, is looking for **transformational therapeutic approaches for sickle cell disease (SCD) and beta-thalassemia**. Specifically, our client is seeking disease modifying approaches using antibody, small molecule, oligo or *in vivo* gene therapy treatment approaches.



Approaches should target the underlying mechanisms of disease to transform outcomes. Approaches that only address the symptoms of sickle cell disease are not of interest, unless they also address the root cause of the disease.

Approaches of Interest:

- Approaches that increase fetal globin levels
- Approaches that correct beta-globin levels
- Strategies to decrease vaso-occlusive crisis/crises (VOCs)

Novel targets to modify the above are of interest, provided they act upstream to correct causal mechanisms of disease.

Out of Scope:

- Approaches that only address symptoms (e.g., iron overload)
- *Ex vivo* cell and gene therapies
- Medical devices
- Biomarkers of disease or screening methods
- Well-established existing targets, or targets that have been genetically linked to increased tumorigenesis

Developmental Stages of Interest:

- Research from preclinical phase through to phase III is of interest
- Opportunities with *in vivo* validation are of highest interest, although opportunities with strong *in vitro* or *ex vivo* validation will still be considered.





Submission Information

Submission of one-page, 200–300-word briefs is encouraged, along with any optional supplementary information e.g. relevant publications. The team encourages including your proposed next steps in developing the research towards commercialization. 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 funded research collaborations and agreements, or licencing of assets.

Opportunities sought

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

Submissions

Please submit relevant, non-confidential opportunities to **Roisin McNally** at rmcnally@usf.edu

Deadline: **30th September 2024 - 4:59 pm GMT**

Have any questions?

Contact **Roisin McNally** at rmcnally@usf.edu