

Accessible and Transformational Therapeutic Approaches for Hereditary Haemorrhagic Telangiectasia (HHT)



Our client, a world leader in therapies for rare genetic diseases, is seeking novel disease modifying therapeutic approaches for hereditary haemorrhagic telangiectasia (HHT) type 1 and type 2. In particular, the team is interested in approaches based on antibody, small molecule, oligo, re-dosable *in vivo* gene therapy or endothelial cell delivery approaches.

Approaches of Interest:

- Increasing endoglin (ENG) or activin receptor-like kinase 1 (ALK1 or ACVRL1) expression/function
- Targeting BMP 9/10 signalling pathways
- Decreasing vascular malformations

Approaches should have demonstrated application to treat HHT, not related conditions involving the above pathways (E.g., cerebral cavernous malformation, pulmonary hypertension).

Out of Scope:

- Approaches for HHT-juvenile polyposis syndrome
- Approaches targeting VEGF pathway
- Targets that have been genetically linked to increased tumorigenesis, osteogenesis or overt toxicity
- Small molecules currently being repurposed (e.g. doxycycline, pazopanib, nintedanib, sirolimus, tacrolimus, pomalidomide, thalidomide, tamoxifen, estrogen/progesterone, bevacizumab, tranexamic acid, somatostatin agonist, anti-TNF)
- Medical devices

Developmental Stages of Interest:

- Opportunities from preclinical research to phase III are within scope
- Opportunities with *in vivo* validation are of highest interest. Opportunities with *in vitro* or *ex vivo* validation will still be considered, please outline your next steps to achieve *in vivo* validation where possible







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

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

Submissions

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

Deadline: **23rd September 2024 - 10:59 pm GMT**

Have any questions?

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