

Genetic Medicine Approaches to Treat Rare Diseases

A biotech rare disease company focused on genetic medicine is looking for novel research and technologies to address rare metabolic diseases, in particular amino acid metabolism diseases and lysosomal storage disorders. Specifically, the team is looking for assets utilising genetic medicine approaches which have potential for development into novel treatments, with the ultimate aim of finding long-term solutions for these conditions.

The company is interested in amino acid metabolism and lysosomal storage disorders, with a particular interest in Phenylketonuria (PKU), Maple syrup urine disease, and Aspartylglucosaminuria (AGU).



Approaches of Interest:

- Techniques of interest include gene editing, gene replacement therapies and RNA modalities.
- Both novel therapeutics and platform technologies are of interest if there is evidence they can be applied to one of the disease areas of interest.
- Platform technologies should be applicable to multiple diseases.
- Approaches based on CRISPR, base editing and prime editing are of high interest.
- Combined therapies e.g. a combination of RNA therapy and gene therapy.

Developmental Stages of Interest:

- Opportunities at basic research phase to phase I are within scope, with a preference for research with proof of concept studies in an animal model.
- Research with *in vitro* validation may be considered if the route to animal model validation is defined.
- Ideally research should have associated IP, or a strategy for this to be established and be unencumbered.
- Late-stage gene therapy assets are also of interest.






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:

Our client is open to a range of collaboration scenarios, with the most appropriate path determined by the needs of the opportunity. Example outcomes include project funding (sponsored research agreements), licensing assets and research collaborations.

Opportunities sought

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

Submissions

Please submit relevant, non-confidential opportunities online via: discover.in-part.com

Deadline: **27th February 2023 - 11:59 pm GMT**

Have any questions?

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