

## Research Programme for Rare and Ultra-Rare Diseases

**Ultragenyx** is a biopharmaceutical company focused on developing novel products for the treatment of rare and ultra-rare diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are typically no approved therapies treating the underlying disease.



Ultragenyx is seeking research focused on **rare and ultra-rare diseases** meeting the following criteria:

- Genetic diseases with substantial unmet medical need
- **Mechanism of therapeutic action supported by strong biology to address the underlying primary defect or pathologic process for disease modification**
- Rare and ultra-rare diseases with prevalence above 1/250,000
- Bone/endocrine, liver/metabolic, neuromuscular and CNS disorders as priority therapeutic areas

### Approaches of Interest

- Approaches may include **biologics (e.g. antibodies, enzyme replacement therapies and peptides), gene therapy approaches or nucleic acid therapeutics**
- Ultragenyx is also open to research focused on haematology, nephrology and cardiology on a case by case basis

### Out of Scope

- Oncology and infectious diseases
- *Ex vivo* gene therapy and cell therapy (e.g. lentivirus vector system)
- Repurposed small molecule drugs

### Stage of Development

Research at basic through clinical phase II stages are of interest, with a particular interest in opportunities with accompanying *in vitro* and/or *in vivo* proof of concept data.





### Opportunity for Collaboration

Ultragenyx 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.

### Submission Information

Submission of one page, 200-300 word briefs are encouraged. In submitting to this campaign, you confirm that your submission contains only non-confidential information.

### 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](https://discover.in-part.com)

Deadline: **22nd November 2021 - 11:59 pm GMT**

#### Have any questions?

Contact our team at [discover@in-part.co.uk](mailto:discover@in-part.co.uk)

