

## Next Generation Gene Regulation Technology Applicable for AAV-Based Gene Therapy

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 interested in research focused on **innovative gene regulation technologies for adeno-associated virus (AAV) based gene therapy**, meeting the following criteria:



### Approaches of Interest

- AAV modalities including standard gene supplementation, but also novel gene editing approaches, improved capsid targeting and regulated gene expression.
- Key focus on gene regulation technology, activating systems, regulatory circuits and target specificity
- Technologies for advancing therapeutic development in such disease areas as the central nervous system, muscle, liver and bone are a priority

### Out of Scope

- Non-AAV viral vector approaches (e.g. lentivirus vector)
- Non-viral vector approaches

### Stage of Development

Research at basic through clinical phase II stages are of interest, in particular 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

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

#### 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)