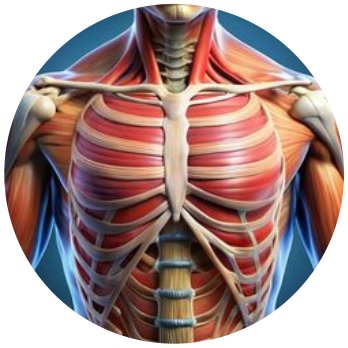
Industry Call for Opportunities

Seeking Novel Solutions to Treat Rare Genetic Skeletal Muscle Diseases



Our client, a global leader in therapies for rare genetic diseases, is looking for novel **approaches to target the primary cause of rare genetic skeletal muscle diseases**, to transform the way patients feel, function, and survive.

The following conditions are of interest: Muscular Dystrophies (Duchenne, Becker, FSHD, Limb-girdle, LAMA2, Emery-Dreifus, Ullrich, Oculopharyngeal) Myotonic Dystrophy Type 1 and 2, Pompe Disease, Nemaline Myopathy, X-linked Centronuclear Myopathy, RYR1-related myopathies and other rare genetic skeletal muscle diseases will be considered.

The team is p**rioritising oligonucleotide (RNAi, siRNA, ASO, saRNA), non-viral gene therapy and small molecule approaches**, but is also interested in gene correction and editing, cell therapy, mRNA and protein replacement and substitution. Novel approaches with demonstrated in vivo proof-of-concepts are of particular interest.

Approaches of Interest:

- The team is interested in approaches that target the primary cause of the genetic disease:
  - Loss-of-function disorders - approaches that restore expression of the damaged gene
  - Gain-of-function disorders - approaches that reduce expression of the abnormal variant that drives pathology
- The team will only consider approaches with strong evidence that their mechanisms-of-action are directly proximal to the primary cause of the genetic disease

Out of Scope:

- Neuromuscular disorders (E.g., ALS, MS)
- Viral gene therapy approaches

Developmental Stages of Interest:

- Novel approaches with demonstrated in vivo proof-of-concepts will be prioritized
- Research at any stage will be considered (early studies through to Phase III and registration)

Submission Information and Opportunity for Collaboration:

Submission of 200–300-word briefs is encouraged, along with any optional supplementary information e.g. relevant publications, patents. The team encourages including your proposed next steps in developing the research towards commercialization and information on the possible impact of the approach such as patient unmet need and addressable patient population. In submitting to this campaign, you confirm that your submission contains only non-confidential information.

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: **5th May 2025 - 10:59 pm GMT**

Have any questions?
Contact our team at discover@in-part.co.uk