Industry Call for Opportunities

Seeking Novel Solutions to Treat Rare Genetic Skeletal Muscle Diseases

Our client, a global leader in the creation and commercialization of pioneering 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.

In particular, the team are focused on Muscular Dystrophy (Duchenne, Becker, Emery-Dreifus, Ullrich, Oculopharyngeal or Facioscapulohumeral) Myotonic Dystrophy Type 1 and 2, Pompe Disease, Nemaline Myopathy, LAMA2 / Congenital Muscular Dystrophy Type 1, X-linked Centronuclear Myopathy, Limb-Girdle Muscular Dystrophy, and RYR1-related myopathies.

The team is interested in a range of approaches including gene correction and editing, cell therapy, viral and nonviral gene therapy, mRNA, oligonucleotides (RNAi, siRNA, ASO, saRNA), small molecules, 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 also 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)
- Lentiviral approaches

Developmental Stages of Interest:

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

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 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.

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