

Research Strategy

The objective of the research strategy is to increase understanding of muscular dystrophy and allied disorders and to support research into and evaluation of potential management and treatment strategies

Specifically the research strategies are to:

1. To further understanding of the basis of normal and abnormal muscle function with the long-term aim of identifying candidate treatments or management strategies for muscular dystrophies

- Progress basic research to have a sufficient understanding of muscular dystrophies and appropriate therapeutic targets with the long-term aim of generating candidate treatments
- Appropriately promote the availability of MDC research grants. Encourage the establishment of consortia and/or collaborations (both nationally and internationally) to enhance the translation of research to the clinic
- Scientific advances of the past decade have opened up the opportunity of exploring new options for the potential treatment of muscular dystrophies. Research into conditions caused by "loss-of-function" mutations will be focussed on developing innovative technologies particularly those with the potential to treat a range of muscular dystrophies:
 - Developing pharmacological interventions (IGFs, myostatin antagonists)
 - Replacing the affected muscle cells (cell transplantation therapies)
 - Restoring a wild-type copy of the gene (gene therapy)
 - Obviating the effect of the mutation (exon skipping)
 - Bypassing the mutation (e.g. upregulate utrophin in DMD)

The potential of these approaches will be evaluated on a regular basis

- Find out what members and potential members, as well as clinicians, regard as current unmet medical needs and aim to establish research proposals in these areas
- Identify marketed treatments which may have therapeutic potential in muscular dystrophies either as treatments or to meet current unmet medical needs of patients

2. To fast-track promising treatments when they are close to clinical trials to ensure a rapid transition from the laboratory into the clinic

- Register treatments for fast-track clinical development (e.g., orphan drug designations) where appropriate, and lobby to make the process more straightforward

3. To progress safe and effective treatments through clinical development to the marketplace in the shortest time

Clinical trials provide the evidence on which treatment decisions are based. Whilst providing funding for large scale clinical trials is currently beyond the scope of the MDC, the MDC is keen to support pilot studies, and work in partnership with other research funders and professional associations, and the pharmaceutical industry to support clinical trials.

- Ensure there are appropriate Clinical Research Centres and facilities including databases of patients in advance of full clinical development
- Work with scientists involved in a wide range of research and care to design innovative clinical research and development strategies for new management approaches and treatments
- Develop close relationships with regulatory authorities to gain an understanding of their requirements for registration in this area