



RESEARCH STRATEGY 2010

IT'S TIME TO STOP WASTING!

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Action Duchenne aims to cure Duchenne. The objective of this research strategy is to increase understanding of Duchenne muscular dystrophy and to support research into, and evaluation of, potential management and treatment strategies.

Specifically the research strategy aims to:

1. Further the understanding of Duchenne muscular dystrophy with the long-term aim of identifying candidate treatments or management strategies for Duchenne.

- Progress basic research to have a sufficient understanding of Duchenne and appropriate therapeutics targets with the long-term aim of generating candidate treatments.
- Appropriately promote the availability of Action Duchenne research grants. Encourage the establishment of consortia and/or collaborations (both nationally and internationally) to enhance the transition 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 Duchenne. Research into developing innovative technologies with the potential to treat Duchenne.
 - Limiting the effect of the mutation (exon skipping)
 - Bypassing the mutation (upregulating Utrophin)
 - Developing pharmacological interventions (myostatin antagonists)
 - Find out what members and potential members, as well as clinicians, regard as current unmet clinical needs and aim to establish research proposals in these areas.
 - Identify marked treatments which may have therapeutic potential in Duchenne, either as treatments or to meet current unmet medical needs of patients.

The potential of these approaches will be evaluated on a regular basis by Action Duchenne's Scientific Advisory Board.

2. 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 straight forward.



3. 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 Action Duchenne, Action Duchenne is keen to support pilot studies and work in partnership with other research funders, professional associations, and the pharmaceutical industry to support clinical trials.