

RESEARCH STRATEGY

**Muscular
Dystrophy UK**

Fighting muscle-wasting conditions





Our vision

Every day Muscular Dystrophy UK is working towards a future with effective treatments and ultimately cures for all muscle-wasting and associated neuromuscular conditions. We believe this as passionately now as we did when we were established 60 years ago.

It is this vision that unites us all and drives our research strategy.

Muscular Dystrophy UK is committed to supporting researchers and clinicians to carry out the best quality research. We continue to invest in areas where we are looking to see change in the near future.



Our broad aims

Deepen our understanding of neuromuscular conditions

We need to deepen our understanding of neuromuscular conditions in order to develop potential treatments and ensure that there is a pipeline for clinical trials. We will continue to support excellent scientists in carrying out first-class, fundamental research into neuromuscular conditions.

Support more studies into ultra-rare conditions

We will work with international funders and charities to ensure that we bring together research skills from across the globe. By collaborating internationally, researchers can reach larger numbers of patients, which will speed up the journey to develop new treatments.

Expand patient access to clinical trials

There are only a limited number of neuromuscular centres currently able to carry out trials, so we will work to ensure that centres across the UK have enough trained staff to support them. This will give more patients across the UK the opportunity to take part in clinical trials.

Encourage new researchers to join the field

To support neuromuscular research in the long term, we need to expand the existing pool of dedicated and talented researchers. We will support and encourage researchers early on in their careers, as well as experienced researchers from other fields, to join the neuromuscular community.

Supporting allied healthcare professionals

Allied healthcare professionals including physiotherapists, orthotists, occupational therapists, speech and language therapists and specialist nursing staff are all key to understanding the impact of a muscle-wasting condition and improving quality of life for those affected. We will provide further opportunities for quality of life research through our grant schemes.

Our key priorities

We held workshops with national and international experts alongside patient representatives to help us identify four key priority areas. These will inform how we will support researchers and promote innovation for the next three years:

To harness
the power of
genetics

To understand
disease
mechanisms

To facilitate
treatment
development

To improve
quality of life

Partnerships and innovation

Research excellence

The theme of innovation and partnership underpins all that we do and enables research to be given every chance to succeed.

Harnessing the power of genetics

- So that:
- we improve our understanding why people with the same condition are affected differently.
 - more people receive a confirmed genetic diagnosis, which will help us better understand the conditions.

Understanding disease mechanisms

- By:
- developing new models that mimic the human condition that can be used to assess the potential of new drugs.
 - investigating what happens inside the body, not only within muscles but also in other organs such as the brain or the gut.

Facilitating drug development

- We will:
- support patient registries and studies that assess how a condition progresses with time (natural history studies), as these are valuable resources for drug companies and other researchers.
 - work across the research community to encourage and support collaboration to speed up the journey to develop new treatments.

Improving quality of life

- We will:
- develop evidence-based interventions that improve the quality of life of people affected by muscle-wasting conditions, putting the patients' knowledge and experiences at the heart of the research agenda.
 - better understand the psychological impact of living with muscle-wasting conditions.
 - investigate the management of fatigue and pain.



Discover more:

To find out more about our research strategy, go to:

[www.musculardystrophyuk.org/
progress-in-research/research-strategy/](http://www.musculardystrophyuk.org/progress-in-research/research-strategy/)

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