

Muscular Dystrophy UK

Organisational strategy

2023 - 2025

Our purpose

We connect our community of more than 110,000 people with one of over 60 muscle wasting and weakening conditions, and all the people around us. So we can all get the healthcare, support and treatments we need to feel good, mentally and physically.

Our mission

- We share expert advice and support to live well now.
- We fund groundbreaking research to understand the different conditions better and lead us to new treatments.
- We work with the NHS towards universal access to specialist healthcare.
- Together, we campaign for people's rights, better understanding, accessibility and access to treatments.

Our values

Stronger together

We believe in the power of community. That the whole is greater than the sum of its parts. Because the more of us who come together, the greater the impact we'll make.

We inspire collaboration across our whole community. Staff and volunteers. Scientists and supporters. Friends and family. Healthcare professionals, other charities and our corporate partners.

We champion equality, diversity and inclusion. Through the way we work and inclusive engagement with the whole community.

Forward thinking

There are over 60 different muscle wasting and weakening conditions. That's more than 110,000 unique individuals. Plus all the people around us. We're here for every single one.

Whoever you are. Wherever you're from. You are our sole focus.

We set ourselves clear targets and measure our impact.

We don't mince our words.

We say what we think.

Here for you

We believe in the power of kindness. We are here for everyone, but we know support isn't one-size-fits-all. We take the time to listen to every individual, so we can tailor our support to you.

We are all about the people who make up our community. The people living with muscle wasting and weakening conditions and those around us. Our friends and family, healthcare professionals and scientists.

We share real life stories at every opportunity. Human authenticity.

Real words. Real images.

Never stop

We've already made advances that would have been unthinkable just 10 years ago, and we are determined to go even further and faster.

We're here for each other. At every stage of life.

We advocate on behalf of our community by amplifying our voices. Raising awareness of the challenges we face and how we'll overcome them.

We have momentum and we're going places. Every day counts.

Our muscles matter

Muscles.

We use them to move.

Walk, eat, smile, cry.

Pump blood around our bodies.

To breathe in and out.

Our muscles matter.

Living with a muscle wasting and weakening condition can be exhausting, stressful and lonely. With endless medical appointments, physiotherapy, treatments and respiratory support.

That's why, for over 60 years, we've been building our community.

We're the leading charity for over 60 muscle wasting and weakening conditions.

Connecting more than 110,000 people affected, and those around us.

So we can all get the healthcare, support – and treatments – we need to feel good, mentally and physically.

Through advice and support, research, campaigning and fundraising.

We support and speak up for each other and our community. Raising awareness of the challenges we face and how we'll overcome them, together. To improve understanding, champion accessibility and accelerate new treatments.

Bringing you together with friends and family, scientists and supporters, staff and volunteers, healthcare professionals and partners. Across the UK.

We have made advances that would have been unthinkable just 10 years ago.

Now, let's go even further, faster.

Together we are stronger.

Together we are Muscular Dystrophy UK.

Delivering change

When we were founded in 1959, research into muscle wasting conditions was largely neglected. Our continued investment into pioneering research and development of clinical support made possible by our supporters, partners and donors, has led to vital progress which, with better care, has improved both quality of life and life-expectancy.

Major highlights of the last ten years:

- We have supported the careers of many early career researchers through our PhD studentships (over 20 in the last decade), clinical research fellowships (five in the last ten years) and through the support of many post-doctoral researchers who have been funded through our project grants.
- We've been instrumental in supporting several patient registries for conditions such as FSHD, myotonic dystrophy, myotubular and centronuclear myopathies as well as collagen VI muscular dystrophies.
- Whenever we can, we work in partnership with other funders to enhance the impact of your donations and to support strategically important areas. Recent examples include a £1M programme with the charity LifeArc to create a £1 million joint fund. The fund will be aimed at accelerating promising research projects from across the UK to develop new treatments for congenital muscular dystrophy. We have also jointly funded a four-year PhD Studentship with Spinal Muscular Atrophy UK.
- Since 2019 we have been funding the MDUK Oxford Neuromuscular Centre, directed by Professor Matthew Wood at the University of Oxford. Its clinical researchers continue to increase the number of clinical trials for children and adults with muscle wasting conditions conducted in Oxford. Teams at the Centre are also leading on projects that will drive positive changes in the field such as natural history studies, and projects that could lead to new treatments for several conditions.

- We have been supporting the world's largest natural history study for Duchenne muscular dystrophy, known as NorthStar, to collect data that will improve the quality of life for children and adults living with the condition. Standards of care for children living with Duchenne were first published in 2010 and revised in 2018. And we funded and supported the development of the first published best practice standards of care for adults living with Duchenne muscular dystrophy in 2021.
- We funded the early research that led Professor Sir Doug Turnbull and the neuromuscular team at the John Walton Muscular Dystrophy Research Centre at Newcastle University to develop IVF mitochondrial donation. For the first time, this technique gives women with maternal mitochondrial disease the choice to have a healthy baby.
- With our support, Professor George Dickson designed, tested and refined a gene therapy for Duchenne muscular dystrophy. This research has progressed significantly in recent years.
- We laid the foundations for exon skipping and, thanks to our groundwork, the first exon skipping drug is now available in the US and we are pressing for it in the UK.

In addition to supporting pioneering research, we have supported the lives of people with muscle wasting conditions thanks to our campaigns, advocacy and support activities. Our campaigning has led to:

- 11 Treatments across five conditions now approved or in the NICE/NHS/SMC approval process.
- Translarna becoming the first drug to be given approval to treat the genetic cause of Duchenne muscular dystrophy, outside of a clinical trial.
- Babies with SMA Type 1 in Scotland, England and Wales being given access to Spinraza, the first effective treatment for the condition.
- Improvements in access to a specialised Changing Places toilet, with over £32m in government grants increasing accessibility in motorway service stations, railway stations and in public places across all but four regions in England.
- Improvements in public transport including more step-free access to stations, and it is now illegal for taxi drivers to refuse to take disabled people or charge them extra.
- More children and adults are taking part in clinical trials at leading muscle centres thanks to our targeted and long-term support.

- Improving access to multi-disciplinary teams support for families through a network of specialist roles, including care advisors, specialist nurses, OTs, physios and consultants, in clinics and centres across the UK.
- More people with muscle wasting conditions now have greater control over their lives through our grant funding towards the cost of vital equipment.
- Thousands of people are better informed, supported locally, and more financially secure, thanks to our expert information, advice and advocacy support.

Continuing change

At the heart of this progress has been people living with muscle wasting conditions alongside work by dedicated researchers and clinicians, the community of health professionals and us.

- We are using research to improve the lives of as many people as possible who are affected by muscle wasting conditions.
- We continue to work to improve NHS services to ensure everyone can access high- quality, specialist care by working in partnership with health professionals to identify and address service challenges; highlight and spread best practice; and upskill health professionals to meet the needs of our community.
- We seek to reach everyone affected through our helpline, links with neuromuscular clinics, virtual and physical events, online forums and peer support network to ensure they get the information, support and care they need so that they can live well and as independently as possible.
- We elevate the voice of people living with muscle wasting conditions to ensure they are treated as equal members of society so they can be part of the workplace, and to remove the barriers to transport and travel that stop them from being fully involved in their communities.

By campaigning, sharing your experience, giving your time or your money, you can join us to make change happen.

Your priorities – our ambitions

The important progress already achieved is heartening, but there is still so much more to do. Our commitment to find treatments for today and ultimately cures through research, and to drive improvements in care and quality of life, remains as strong today as ever.

Creating the strategy

This strategy was created during a period of uncertainty, there was a global pandemic, cost of living crisis and a positive change in the pipeline for treatments for people living with muscle wasting conditions that needed to continue. During a two-year period, we worked alongside people directly affected by muscle wasting conditions, the healthcare services and researchers to understand the priorities and to provide support; to talk, listen, measure and consider the changing environment to create this strategy. Our community makes us stronger, we are committed to making sure we work together in setting our priorities and the approach we take, from board level, stakeholders, committees, partners, employees and volunteers, all the way to operations on the ground.

We do this by:

- listening to people and gathering evidence to better understand their experiences and to ensure the voice of people affected by muscle wasting conditions is heard and acted upon
- understanding people's experience of Muscular Dystrophy UK, so that we can improve our provision of information, advice and support services, both nationally and locally
- ensuring people with muscle wasting conditions help set our strategy and drive our work as board members, being part of our committees nationally as well as volunteers locally
- enabling people to have a choice and a voice in their local communities through actively supporting inclusion in local decision-making and community planning
- actively engaging with the research community, being a member of the AMRC (Association of Medical Research Charities) and ENMC (European Neuromuscular Centre) to elevate the needs of our community and accelerate the opportunities in the UK and globally.

Two of the key reports we wrote during this period can be found on our website:

- The impact of COVID-19 and the future of care for people with a muscle wasting condition
- The Impact of rising costs on people living with a muscle wasting condition

Transforming lives through research

Our vision

Every day we're 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 in 1959.

It is this vision that unites us all and continues to drive our research strategy.

We fund pioneering research for better treatments to improve people's lives today, and to transform those of future generations. Every day scientists are gaining new knowledge of muscle wasting conditions, so there are always exciting new treatments on the horizon.

The theme of innovation and partnership underpins all that we do and enables research to be given every chance to succeed. By supporting research in the UK and across the world, we link researchers and research projects, accelerating scientific progress and helping us to improve lives as quickly as possible.

Many conditions are regarded as very rare, but research is working towards new treatment possibilities all the time. For example, scientists are building on what they have learnt from developing therapies for conditions such as Duchenne muscular dystrophy (DMD) and spinal muscular atrophy. Some of these findings are now taking us towards more targeted treatments for DMD, collagen-VI muscular dystrophy and mitochondrial diseases.

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 very 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.
- Facilitate clinical trial readiness

- There is a growth in potential treatments becoming available for neuromuscular conditions. It is, therefore, vital that researchers have enough information about the symptoms and biology (natural history) to design clinical trials and show the regulators that a treatment is beneficial. To help facilitate clinical trial readiness, will support studies that help us understand the natural history of conditions and provide insight into the best outcomes to measure to show treatment effectiveness. In addition,
- 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

The priorities that we set alongside people with lived experience of muscle wasting conditions in 2019 remain relevant today:

- To harness the power of genetics
- To understand disease mechanisms
- To facilitate treatment development
- To improve quality of life

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 treatment 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, pain and other aspects that impact on the quality of life experienced by people living with muscle wasting conditions.

Investing in the future we will:

- continue to invest money in high-quality research to understand the underlying causes of muscle wasting conditions, and to develop potential treatments, always making sure our investment has maximum impact. Through our annual grant round we currently support many grants that help us deliver our research strategy. However, there are aspects that can be accelerated if we make some strategically placed investments.
- encourage scientists with new ideas and new technologies to come into this area of research, building on existing scientific study to develop a new generation of therapies, with the ultimate goal of being able to treat all people, whatever their muscle-wasting condition.
- use seed funding to drive innovative research to enhance the quality of life for people living with muscle wasting conditions.

Driving improvement in health and social care

Access to Treatments

When we produced our last strategy there was only one treatment available beyond access through clinical trials for one form of Duchenne muscular dystrophy. By the end of that strategy period we were active on the appraisal or roll-out of 11 treatments across five of the conditions we cover. We sought to improve the landscape for rare disease treatment approval through engagement in major policy developments such as the NICE methods review, while shepherding these 11 treatments through their individual appraisal processes. Over the next three years we anticipate even more treatments coming forward for appraisal, and so this role will be required of us more than ever before.

But as treatments become available, smooth access to them is not guaranteed and we must also work to help highlight and address the barriers that are faced by neuromuscular services to their rollout.

Supporting professionals

We rely on well trained and supported NHS staff to meet the needs of our community – but we know that the NHS isn't able to provide this to the level that neuromuscular services aspire; or that our community requires. That's why we continue to provide training and networking resources and opportunities to both neuromuscular specialist healthcare providers and community health professionals so that they can provide improved care and ensure more people affected by muscle wasting conditions receive the support they need.

Improving access to NHS specialist support

Multi-disciplinary care is essential to the effective management of muscle wasting conditions. It brings together the expertise of professionals from a wide range of disciplines in one location to provide co-ordinated, patient-centered care. Individuals and families want to access a specialist multi-disciplinary team to help manage their condition, regardless of where they live in the UK. We seek to facilitate this through our identification of service gaps and challenges and partnership working with neuromuscular services and networks to overcome these through targeted engagement with commissioners and decision makers .

Our objectives for driving improvement in health and social care

Fast track to treatments

We will:

- help meet the growing demand for clinical trials through our investment in the MDUK Oxford University Neuromuscular Centre, which promotes the translation of scientific research into clinical trials; improve the clinical trials infrastructure for neuromuscular conditions; press for more staff at other neuromuscular centres UK-wide to grow capacity to deliver trials and treatments
- ensure that clinical trials are designed robustly and take into account measures that are important to people with muscle wasting conditions; work to ensure that individuals and families affected have the opportunity to take part in clinical trials wherever they live in UK
- harness the skills and expertise of volunteers to provide support and information to families on clinical trials.
 - Work collaboratively with health professionals, pharma companies, NHSE, NICE, and other patient organisations on pre-appraisal access to treatments for neuromuscular conditions, including Early Access to Medicines Schemes.
 - Engage in and work collaboratively on all NICE and SMC appraisal processes for treatments for neuromuscular conditions.
 - Engagement in the roll-out of relevant Managed Access Agreements
 - Highlight and push for the resources needed by neuromuscular services to roll-out new treatments
 - Where possible, share our learnings and work in collaboration to seek system change.

Supporting professionals

We will:

- work in partnership to ensure that health professionals better understand muscle wasting conditions and their impact on people's lives
- improve access to support for people living with a muscle wasting condition from a comprehensive multi-disciplinary team that includes a neuromuscular care advisor, specialist neuromuscular physiotherapy and psychological support.

- upskill both specialist and non-specialist neuromuscular health professionals through our suite of e-learning modules, specialist health professional conferences and virtual seminars
- work in partnership with neuromuscular services to secure additional support and resource so that people living with muscle wasting conditions have the best possible access to the comprehensive care and support they need.
- enable networking of neuromuscular specialist health professionals, the dissemination of best practice and the identification of common concerns and challenges through our Regional Neuromuscular Networks and through engagement with the two NHS run Regional Neuromuscular Networks.
- Support neuromuscular services to navigate and engage in the development and roll-out of Integrated Care Systems, Rare Disease Framework action plans and other NHS reforms
- Use the findings of the audit of neuromuscular services to recognise and promote excellent delivery of care and support.
- Develop our most comprehensive picture of neuromuscular services across the UK through the relaunch of our audit of neuromuscular services and produce a national assessment of the support that is being provided by the NHS.
- Use the findings of the audit and intelligence gathered from Regional Neuromuscular Networks to identify local service challenges and work in partnership with services to address them; and to identify common themes shared across services that need to be addressed at a system-wide level.
- Seek to ensure that the development and roll-out of Integrated Care Systems, Rare Disease Framework action plans and other NHS reforms benefit both NHS neuromuscular services and the people who use them.

Improving access to NHS specialist support. We will:

- Continue to drive improvement in specialist neuromuscular care across the UK.
- Develop our most comprehensive picture of neuromuscular services across the UK through the relaunch of our audit of neuromuscular services and produce a national assessment of the support that is being provided by the NHS.
- Use the findings of the audit and intelligence gathered from Regional Neuromuscular Networks to identify local service challenges and work in

partnership with services to address them; and to identify common themes shared across services that need to be addressed at a system-wide level.

- Seek to ensure that the development and roll-out of Integrated Care Systems, Rare Disease Framework action plans and other NHS reforms benefit both NHS neuromuscular services and the people who use them

Living well with muscle wasting conditions

We are here for all people and their families affected by a muscle wasting condition – whether awaiting a diagnosis, recently diagnosed or living with a condition.

Mental health matters

Being diagnosed with a muscle wasting condition means adjusting to a new and unexpected reality. Muscle wasting conditions can affect every aspect of life and many people tell us they feel isolated because of their condition. Others tell us of their anxiety or depression as they adjust to their diagnosis. The impact goes beyond those who have a muscle wasting condition; families and carers also live with its effects. We want to see better support for people's psychological needs from the point of diagnosis and at every stage thereafter.

Supporting independent living

We help people to live well with their condition by providing information, practical advice, advocacy and support on the phone, online, through our specialist information and advocacy officers, through our local Muscle Groups and through events and our virtual Muscles Matters seminar series.

People affected by muscle wasting conditions can find it difficult to access the benefits to which they are entitled. We will ensure that they get the support they need and help fight on their behalf where necessary, seeking to overturn unfair or incorrect decisions.

Cost of living

People across the UK are facing an uncertain economic landscape, with everyone experiencing rising prices and an increase in the cost of living. But for our community the impact of this can be particularly profound as we heat our homes to stay well; keep the power on to use and charge essential mobility equipment and to keep life-preserving ventilation equipment working; fill up our cars or use public transport to attend multiple healthcare appointments; and purchase

already more costly specialist food and clothing. We want to ensure that there is targeted support for our community and that everyone is aware of and able to access support that may be available to them.

Accessible communities

It is vital that we are able to access our communities so that we can go to work, do the things we enjoy and remain connected to our friends and family. We support two key elements that help to make this a reality – access to essential powered mobility equipment through our JPT grant giving programme and access to adequate toilet facilities.

Many people living with a muscle wasting condition often need extra equipment and space to allow them to use the toilet safely and comfortably. These needs are met by Changing Places toilets. Since 2017 we have co-chaired the Changing Places Consortium. In that time, the number of Changing Places toilets available across the UK has risen from 1,036 to 1,875 – and there are almost another 680 due to become available over the next two years through Government funding schemes that we have helped to secure and administer.

Our objectives for living well with muscle wasting conditions

Mental health matters

We will:

- seek to improve access to NHS psychology services, use our influence to boost funding towards these services being a key part of the multi-disciplinary team at muscle centres
- work with health professionals so they are trained to understand and help support the mental health needs of people living with muscle wasting conditions

Supporting independent living

We will:

- have high-quality accessible support for everyone affected by muscle wasting conditions, on the phone, online and face-to-face, so that through our signposting, training, advocacy and co-ordination, they will be able to access wider health, housing, education and care services.
- strengthen our links with neuromuscular services so that people have easy access to our support

- deliver regional physical events to help people come together and access our support
- continue delivery of our Muscles Matter virtual seminar series so that people can better understand their specific condition and the support that is available to them to live well
- ensure that that our support is fully inclusive and is accessed by all who need it.

Information

We will:

- update our wide range of information resources and ensure that they are fully accessible to our community.

Cost of living

We will:

- continue to highlight the specific needs of our community and work in partnership with other health and disability groups to secure targeted support for our community
- keep our community up to date on sources of support that are available and how to access them

Accessible communities

We will:

- Provide grants to members of our community for essential powered mobility equipment through our JPT grant giving programme
- continue – in partnership with the Changing Places consortium – to press for more fully- equipped Changing Places toilet facilities across the UK
- continue to support the delivery of Government funding schemes
- work with Changing Places partners to identify a sustainable future for the oversight of Changing Places toilets

Raising money for better lives

This strategy is being prepared as the economy reaches the highest interest rates seen in the UK for over 30 years. Concerns around how this will continue to increase demand on services equal the concerns around the ability for individuals and businesses to donate and essentially fund the activities our community needs.

We will continue to raise funds to ensure more people with muscle wasting conditions can enjoy better lives every day. During the pandemic we were charity leaders in fundraising innovation, and our community did everything they could to help us meet that demand. We will continue to innovate, work closely with our community and raise funds in a way that builds public trust in what charities do and how they do it.

We will encourage and support those who want to join us, using their generous offer of time, talent and money, in the most effective way possible. We will make sure that everyone who wants to support us, including volunteers and campaigners, can do so alongside those who want to change their communities.

Above all, we will ensure all those who support us have a good experience , and know how grateful we are – because, without you, we couldn't do what we do.

Thank you.