



IMPROVING LIVES

SEARCHING FOR A CURE

# REBUILD, REFOCUS, AND REINVENT

## ACTION DUCHENNE STRATEGY 2024-2026

*Our vision to build “a world where lives are no longer limited by Duchenne muscular dystrophy”*



<b>MESSAGE FROM OUR TREASURER &amp; CHIEF EXECUTIVE OFFICER</b>	<b>3</b>
<b>WHAT IS DUCHENNE</b>	<b>4</b>
<b>WHO WE ARE</b>	<b>4</b>
Our values	4
Governance	5
Our Approach	5
Financials	6
<b>OUR STRATEGY 2024-2026</b>	<b>7</b>
Organisational priorities	7
Instil 'All-through Support' Programme Strategy	7
Instil Research and Science Education Programme Strategy	8
Instil Fundraising and Community Engagement Strategy	9
<b>THE FUTURE: OUR 10 YEAR AMBITION</b>	<b>10</b>
<b>TESTIMONIALS</b>	<b>11</b>

## MESSAGE FROM OUR TREASURER & CHIEF EXECUTIVE OFFICER

Action Duchenne is a registered charity founded in 2001 that aims to support every child, young person, adult, and family throughout their Duchenne journey. Our activities are focused on supporting parents through diagnosis and helping families make informed decisions, building a community by uniting and supporting families, educating about Duchenne, and raising the profile of the condition.

We have a clear vision: **a world where lives are no longer limited by Duchenne muscular dystrophy**. This strategy is vital in removing those limits.

93.8p in every £1 readied is spent on our charitable activities, to achieve our vision by:

1. **supporting Duchenne families all-through their journey**, building a community by uniting and supporting families, educating about Duchenne, and raising the profile of the condition.
2. **working with the NHS** to improve care and provide their patients with the earliest possible access to emerging and approved medicines.
3. **funding research** into potential therapeutic strategies for everybody living with Duchenne.
4. **striving for a more inclusive society**, promoting the importance of human equality, day-to-day acceptance of disability, and accessibility.

Action Duchenne is a community-led charity. Our continued efforts to build constructive relationships with our partner organisations, regulating bodies, pharmaceutical companies, and funders will enable us to support the development of more treatments.

As science advances, and more effective treatments are developing, the wider effects of Duchenne are changing and people are living for longer with the condition. With an increased need for support, we have identified opportunities to bring our 'All-through Support' to the entire community, and this will be a major area of focus for Action Duchenne.

We look forward to keeping you updated with regular stories, updates, and highlights on our website [www.actionduchenne.org](http://www.actionduchenne.org)



**Roger Cockerton**  
Treasurer

**Florence Boulton**  
Chief Executive Officer

# WHAT IS DUCHENNE

Duchenne muscular dystrophy is a rare, life-limiting genetic condition caused by mutations in the dystrophin gene, which prevents the production of a vital muscle protein called dystrophin. The lack of dystrophin makes muscles more vulnerable to damage and leads to muscle wasting over time. Duchenne is usually diagnosed around the age of 4, and people living with the condition experience progressive muscle weakness. They typically need to use a powered wheelchair from their early teens, and since the heart and respiratory muscles are affected most will require a ventilator in their twenties. Life expectancy is around 30 years, and there is no cure. However, with better technology, awareness and improvements in the standards of care we are seeing people with Duchenne living for longer and more fulfilled lives.

# WHO WE ARE

Action Duchenne is a UK registered charity, was established in 2001 to support children, young people, adults, and their families living with Duchenne muscular dystrophy. It is a community-led charity, proud to work with partner organisations to address the unmet needs of the community through designing programmes and services that maximise resources.

Action Duchenne has a very clear vision:

**“A world where lives are no longer limited by Duchenne muscular dystrophy”**

## Our values

### **Commitment**

Bringing together staff, trustees, and patrons, along with a supportive network of volunteers is how we remain committed to supporting those impacted by Duchenne.

### **Rigour**

Governance procedures, policies, and processes are well controlled and in line with regulatory frameworks and best practices; quality evaluation and reporting are essential to our approach.

### **Collaboration**

We work closely with trustees, stakeholders, national and international partner organisations to achieve goals.

### **Innovation**

Projects and programmes are created based on the feedback we receive from the families we support; we seek to satisfy unmet needs for individuals and families living with Duchenne.

### **Advocacy**

We advocate within the Duchenne community, as well as among pharmaceutical, health and social care sectors for policies that support individuals with Duchenne, and their families.

### **Proactiveness**

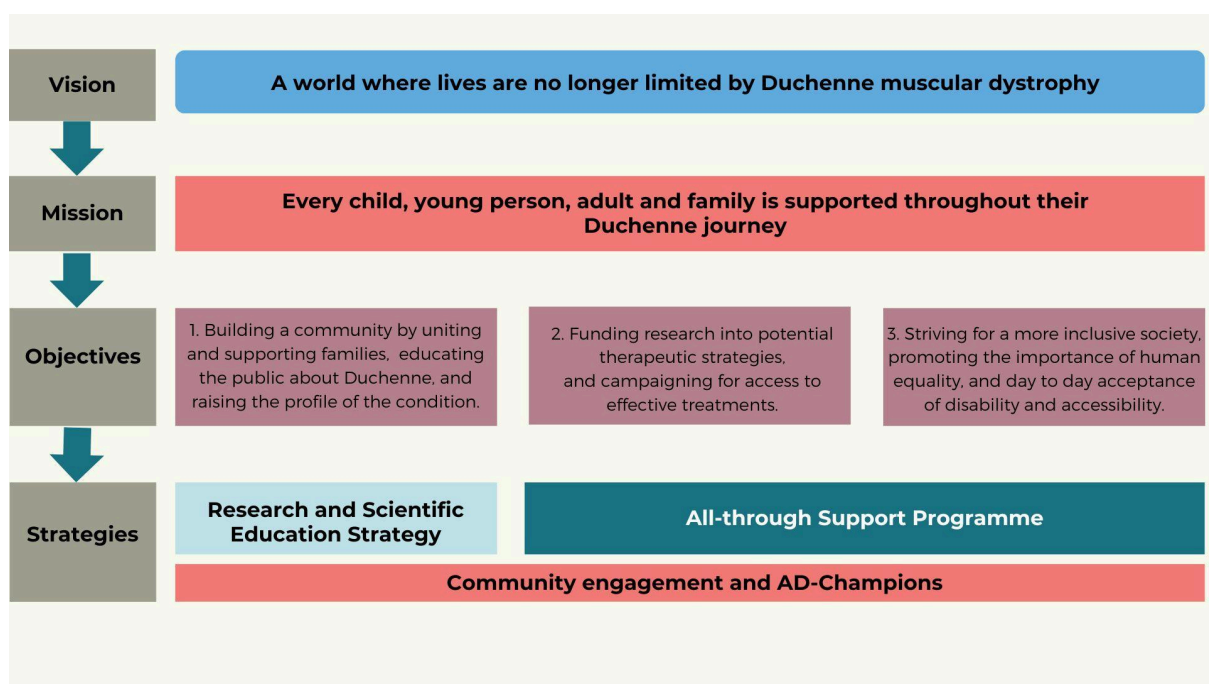
We focus on forging partnerships with funders, organisations, corporates, and individuals to drive fundraising, revenue, and find new income streams.



## Governance

Action Duchenne is a UK-based registered charity guided by a dedicated volunteer Board of Trustees. Our expertise is driven by the lived experience of our team: half of our staff and Trustees have a direct connection to Duchenne. We combine our experience with expertise from across the business, research, and charity sectors to give us an unparalleled ability to advocate for change. We have an in-depth understanding of families' Duchenne journeys, and the leadership and expertise to deliver complex projects to efficiently support those journeys.

## Our Approach



> Action Duchenne 'All-through Support' programme focuses on:

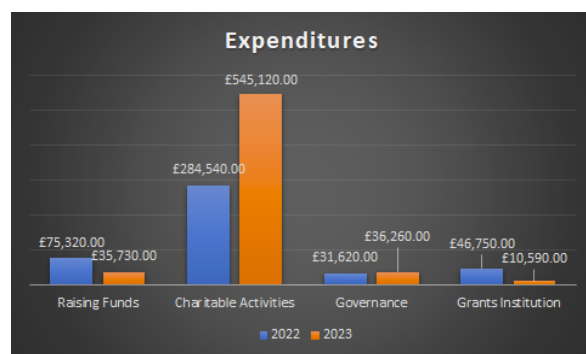
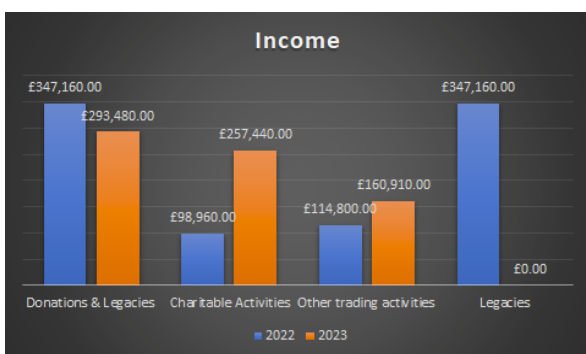
- 1) supporting children, young people, and adults living with Duchenne and their families at every stage of their Duchenne journey
- 2) supporting families dealing with anticipatory grief after diagnosis; and
- 3) fostering the qualities of resilience, determination, independence, and adaptability



## Financials

Our work to date has seen over £13 million in ground-breaking research, educational programmes and campaigns, collaborating with more than 15 organisations globally to fund projects across four continents.

In 2023, the charity's income reached £712k. We allocate **98.3p out of every £1 raised** to our charitable activities: scientific research, education, and supporting families.



## OUR STRATEGY 2024-2026

Our vision: '**a world where lives are no longer limited by Duchenne muscular dystrophy**', outlines our ambitions to transform our community. We have understood our purpose, and communicating our vision constantly and consistently has been vital, in particular during periods of changes. To achieve this vision, and after reviewing Action Duchenne's current Strategic Plan and Operational Plan, the proposed strategy aims to **Rebuild, Refocus, and Reinvent** Action Duchenne and its activities, to further expand our reach, and to always strive to answer the unmet needs of the families we support.

Therefore, our strategy focuses primarily around the organisational level, while also addressing our three organisational priorities, as outlined below.

### Organisational priorities

At the organisational level, three important aims will be prioritised for the 2024-2025 period. These will be supported by ensuring that Action Duchenne has the right people, tools, and infrastructure to support a greater number of Duchenne families, from a variety of diverse backgrounds. We will:

### Instil 'All-through Support' Programme Strategy

While we hope that research and clinical trials will one day lead to a treatment, or cure, Action Duchenne believes it is vital to support families living with the condition today. In 2024/25, we will continue focusing on the following for each component of the 'All-through Support' programme:

#### > Supporting parents through diagnosis

- Improving the referral pathway to Action Duchenne by developing partnership with clinics and specialised hospitals.
- Supporting families for the first 12-18 months of being newly diagnosed.

#### > Helping families make informed decisions

- Increasing the number of Science education workshops across the UK and Ireland.
- Supporting patient experts during the appraisal process to ensure that the voice of the community is effectively heard and can drive impactful change.

#### > Supporting families

- Facilitating regional support groups and offering families access to group counselling to complement the 1:1 and group support contact we already provide.
- Growing the network and empowering families to take on the management and organisation of their group (facebook, regional meet-ups etc.).

#### > Supporting young people transitioning to secondary school and to adulthood

- Supporting individuals transitioning from primary to secondary school, and to adulthood.

- Providing professionally-led online and in-person skills training that empowers young people and adults living with Duchenne to seize control of their lives and have positive role models in their lives.
- Exploring topics based on the needs expressed by young people living with Duchenne
- Offering more regional day programmes, as opportunities to meet with peers in person.

#### > **Support through end of life and bereavement**

- Providing holistic support to help families gain a better understanding of end-of-life journeys and have the difficult but important conversations about end-of-life care. This is to help individuals and families make informed decisions about care.
- Working with other bereavement support organisations for signposting and keeping our bereavement web pages up to date.

## Instil Research and Science Education Programme Strategy

**Our funding support extended to various research initiatives, ranging from Genome Editing and Exon Skipping to enhancing standards of care for bone health and identifying the needs for DMD caregivers all aimed at providing better support for Duchenne patients and their families.**

We will campaign for access to therapies for the Duchenne community and will do this by supporting the best possible multidisciplinary standards of care, across the age spectrum, providing news alerts to the Duchenne community about results, and offering updates from on-going clinical trials.

Our commitment also extends to educating families about Duchenne science to empower them to make informed decisions regarding the management of the condition. We believe that by fostering a deeper understanding of the scientific aspects of Duchenne muscular dystrophy (DMD), families can better navigate treatment options and actively participate in discussions with healthcare providers. Through targeted educational initiatives, we aim to equip families with the knowledge and resources necessary to advocate for the best possible care and access to therapies for their loved ones affected by DMD. By bridging the gap between scientific research and patient care, we strive to ensure that every family impacted by Duchenne receives the support and information they need in order to make the most informed decisions for their journey with the condition.





Fully committed to our goal to make effective treatments available to everyone living with Duchenne muscular dystrophy, our research strategy in the upcoming period will focus on:

**> Supporting innovation**

- Continuing to engage with experts in the Duchenne field to review the emerging therapeutic advances;
- Co-funding novel ideas by Duchenne healthcare professionals who apply. Those awarded funding will be expected to publish their results;
- Utilising our conference platform to showcase emerging scientific advancements by inviting experts from around the world to present Duchenne science and discuss the relevant clinical trials in the field.
- Strategically investing in the capacity that underpins the UK DMD Registry. We see collaboration between the charities in this area as vital, to the aim of being at the forefront of facilitating clinical trials and other transformative research.

**> Standards of care**

- Attending national and international conferences, scientific meetings, and educational workshops to be up to date and equipped with the relevant information in the Duchenne research field;
- Continuing to support pharmaceutical companies by participating in their studies to understand the community's response to their therapeutic direction;
- Attending in-person meetings to discuss the drug regulatory application strategy, and taking part in patient-engagement events to speak on behalf of the Duchenne community;



- Working closely with NICE and partner organisations to advocate for the right to access Duchenne therapies;
- Recording bite-size scientific information videos to discuss the latest in Duchenne science; and clinical development of treatments.

## Instil Fundraising and Community Engagement Strategy

Action Duchenne is a small charity that works with a niche cause, its resources are limited both in terms of staff time and its potential to invest in new fundraising initiatives. However, we are also an ambitious organisation and committed to developing the income streams necessary to meet the needs of the Duchenne community. As such our strategy is to:

### > Play to our strengths:

One of our greatest assets is the Duchenne Community. Families and friends have demonstrated their unwavering support to our work; willing to donate, fundraise and advocate for Action Duchenne. We plan to fully realise the income generating potential within the Duchenne community by:

- Better understanding what motivates our community to donate and fundraise for us
- Strengthening our fundraising messages both in terms of content and format
- Ensuring that our messages are targeted to resonate more effectively with their recipients
- Exploring opportunities to develop family funds as a way of offering direct support to families whilst at the same time generating income for Action Duchenne.
- Finding new ways to reach all corners of our community
- Developing a high profile fundraising campaign (Challenge 79) around World Duchenne Awareness Day

### > Prioritise revenue streams with the greatest earning potential and the highest return on investment

The All-through Support Programme has demonstrated itself to be fundable with grants from the Lottery and corporate companies. Furthermore, we have the in-house skills to develop compelling grant applications. We will expand on this by:

- Focusing on trusts and foundations with an interest in supporting children, health issues, and disability.
- Seeking high value individual/regular donations, high value fundraising events (such as gala balls) and corporate partnerships (eg. Charity of the Year affiliations).
- Continuing to develop a strong evidence base for the impact that our programmes have in order to strengthen our case for support.
- Developing a high profile fundraising campaign (Challenge 79) around UN recognised World Duchenne Awareness Day

### > Integrate fundraising across all our operations

We see fundraising as an important way to strengthen our relationship with the people in our Duchenne community. We do not want conversations about fundraising to be separate from the conversations that we have with them when offering support. Therefore, we will

encourage our support officers to be actively involved in fundraising as advocates for our community.



## THE FUTURE: OUR 10 YEAR AMBITION

Action Duchenne knows that it will take a coordinated and sustained national and international effort to fulfil our vision. Our long-term success will be defined by:

- The availability of better treatments
- Improved care and life expectancy
- Families having the knowledge they need
- People gaining acceptance more quickly than they do today
- A significantly increased science education and research programme
- Appropriate support pathways for families starting at diagnosis and continuing throughout their lives
- Opportunities for people living with Duchenne don't have to be sought, they just exist - university, jobs etc.
- Much wider reach for all our projects - nationally and internationally
- Respite centre(s) available for families

Bringing our whole community together - creating shared values with our partner organisations has been fundamental to the way we deliver our work at Action Duchenne. We believe that our charity can only be successful in the long term by creating value for both partner organisations and the community -putting people in the lead to help define and refine our work and our role.

## TESTIMONIALS

As an organisation, the voices and experiences of individuals and families living with Duchenne are at the centre of our work. To this end, a key way we measure the effectiveness of our programmes is through the testimonials that beneficiaries offer us each year. To conclude our strategy, we highlight testimonials which speak to the three core objectives guiding our organisation.

### **Building a community by uniting and supporting families**

*“I left the workshop feeling like I was part of a community and have people I can turn to if I need help and support”* Workshop Participant

*“Here I have met other Dads who are on the same journey. We sometimes talk about this journey and sometimes we just chat; but either way I know I am chatting with Dads that get it. I have already begun to forge lasting friendships through this forum and am very grateful for that.”* Jamie, Duchenne parent

*“The science on tour has given me an understanding of the condition in a language that I, not being a scientist, can understand. I like the friendly atmosphere and the chance to ask questions. I have also found it really helpful to meet other parents and chat about things. Please keep up the good work, I appreciate all the effort you go to.”* Science Education workshop attendee

### **Developing effective treatments by funding research, and advocating for access to treatments.**

*“As a patient organisation we have the power to make change happen.... Our experience is powerful – we can make change happen when we speak from our experience to policymakers. Our voice is crucial for researchers and policymakers, because it provides evidence of the need for change.”* Kathy, Duchenne parent and campaigner

*“Specialist advice, and advocacy specific to the current difficulties we were experiencing. A shoulder to cry on, and the chance to build friendship- friendships that matter so much when times are tough!”* Ben, Duchenne parent



## Striving for a more inclusive society

*"It is so encouraging to see what I can do despite having Duchenne Muscular Dystrophy"*  
‘Yes I Can’ Programme Participant

*"It was a hugely helpful day for us in clarifying a lot about our son's potential future and reassuring us that there is more hope than we imagined"* - Science Education Workshop Participant

