Conquering Duchenne Muscular Dystrophy
Case for Support

Alex's Wish is an award-winning charity based in the heart of the East Midlands. Raising funds and awareness to conquer an aggressive, fatal muscle wasting disease called Duchenne Muscular Dystrophy which affects 1 in every 3,500 boys born.

Every single week, two families receive the same devastating news that Charity Founder, Emma Hallam did when her son Alex was diagnosed back in 2010. Having learnt not enough funding was going into conquering Duchenne, Alex's Wish was established in 2012 with a mission to eradicate the disease.

With no cure at present, and limited treatment options available in the UK to help slow down muscle wasting; Alex's Wish is helping to drive transformative change and help re-write the history books of this disease.

Our Strategic Focus to Funding.

- Funding early-stage medical research to drive innovative treatments and technologies. This includes building a strong portfolio including therapeutics, biomarkers, devices, and technologies as well as clinical and translational drug development. It is important we consider all disease stages.
- Funding clinical posts (through the DMD Hub) increasing opportunities for participating in clinical trials in the UK.
- Cutting edge technologies to prolong strength, dignity, and independence.
- Clinical trials to bring new treatments to market, improving care standards in the UK. We only ever invest in the best science to improve/extend the lives of those affected.
- Projects to help improving care standards (DMD Care UK) ensuring everyone living with Duchenne in the UK gets the best standards of care.

Research focus and desired outcomes.

Medium Term Outcomes (1-2 years)

- More treatments in clinical development for Duchenne (all stages).
- Novel and improved clinical trial design and measures.
- Better access to data and better datasets.
- More technology innovation projects in the pipeline.

Long-term Outcomes (2-5 years)

- Patients have early access to transformative treatments.
- More successful clinical trials.
- Improved quality of life for all Duchenne patients through innovative solutions and technologies.

This generation will either be the last to die, or the first to survive – and that is how close we are getting. With your help, we will get there sooner.

Collaboration.

Alex's Wish has an army of supporters, majority who have followed our journey since the start. Our brand identity has grown as an established charity in the Leicestershire and surrounding areas, and we have won several awards for our work. Our personal story and identity are what resonates with supporters.

We collaborate with Duchenne UK as one of their Charity Partners. Both charities are run by mothers who have children living with Duchenne. We understand first-hand the challenges affecting families, as we live with this condition every single day. We know we are stronger together and we do not duplicate effort.

Both charities share the same mission and are driving innovative change to help conquer Duchenne for this generation. Both charities collaborate and fund projects together. Duchenne UK generate support from the wider Duchenne community, and collaborates with scientists and the pharmaceutical industry, bringing them together to share knowledge. Also working with health services so that patients get the care they need.

We forge partnerships with the business community and the wider public in the community to drive additional funding and awareness from an audience that ordinarily would have never heard about Duchenne. We also collaborate with

foundations and groups including The Brothers Trust, and Miss Great Britain, amongst many others.

Our Mission and Vision.

Our mission to drive research and clinical trials to find transformative treatments and technologies for Duchenne. Our vision is to end Duchenne and for this generation of children to benefit.

We only ever invest in projects which have been through a leading scientific advisory board who evaluate proposals against strict success criteria. This ensures that our funds are only invested in the most promising projects and clinical trials that are both of high scientific quality and have a clear route to market.

About Duchenne Muscular Dystrophy.

Duchenne is a genetic disease affecting the X chromosome that causes muscle weakness and wasting. It mainly affects boys. Children born with Duchenne have a fault known as a mutation, in their dystrophin gene which means that they cannot produce dystrophin, which is a protein that protects muscles. Without dystrophin, muscles get damaged more easily and so muscle strength and function are weakened.

Often the earliest indication that a child may have Duchenne is their difficulty in getting up from the floor, walking or running. Gradually, the child's other muscles will begin to weaken as well. Without dystrophin, muscles become inflamed over time. As the muscles try to repair themselves, fatty and fibrous material builds up in the muscle and causes them to stop working altogether. This is called fibrosis and causes the muscles to become rigid and hard.

Advances in research and knowledge of the disease are increasing life expectancy. Now, more young adults with Duchenne lead full lives – going to university, having relationships, and living independently. However, those living with Duchenne do still have a reduced life expectancy. We lose boys as young as 11 years old to this disease. Duchenne is a life-changer, it affects every single day of your life as you must be prepared for ever-changing challenges that affect your child's day to day activities.

Duchenne is a progressive, life-limiting muscle wasting disease – it is relentless – affecting every single muscle in the body – including the heart and lungs.

One day your child may not be able to walk, may not be able to lift a cup to their mouth, may not be able to play around at their friend's house anymore. It is overwhelming at times and affects how you live your life at every level. It affects siblings too as often parents will spend much of their time caring for their child with Duchenne. It requires a multi-disciplinary medical team to support their ever-changing needs.

It's incredibly stressful for the families affected as they live in fear of the next stage of progression and wonder how they are going to cope. It leaves those with it in a wheelchair by around the ages of 8-12. Muscle strength continues to decline affecting the arms, neck, and torso by teens. Lives are cut short due to heart and respiratory complications.

Duchenne in numbers.

- 1 of the most serious genetic diseases in children.
- 100% fatal.
- 1 in every 3,500 boys born will have Duchenne.
- 2,500 boys affected in the UK.
- 300,000 boys affected worldwide.
- 2 families per week receive diagnosis in the UK.
- 90% of boys will be wheelchairs by the age of 12.
- 1% is the number of girls affected.
- 25 is the average age boys will lose their lives, but with better care standards this is improving and, we are seeing men living longer. But in our eyes, not long enough.

Our Urgent Need for Funding.

Duchenne current has a high unmet medical need.

We must provide substantial funding to several clinical trials and research projects that will provide a catalyst to drive significant investment from biotech and pharmaceutical companies.

A significant factor in getting treatments out of the laboratory and into clinic is funding! Rare diseases get very little funding from the Government, so charities like Alex's Wish must do what they can to change the outcomes of this disease. It is likely

multiple drugs / a combination of therapies will be needed to repair, restore, or replace the missing dystrophin protein.

This generation of children could be the ones to survive this condition – but only if action is taken now. Alex is now 17 years old, and time is not on our side if we want new treatments to help save him and others of similar age. Without funding, our vision will not happen.

Our urgent need for funding is the sheer fact that many opportunities now exist and the research landscape for Duchenne has never been more prevalent. Never have we seen so many clinical trials available to those families affected. We need to see this work through to the end when effective treatments will be available.

Our Impact.

We are seeing great progress, thanks to the work charities like us have done. Since being in this space since Alex diagnosis, we are starting to see new treatments to help slow down disease now emerging. However, we must continue to fund best science to bring more effective treatments to market as well as find a cure for all.

- We invest the most promising scientific research available to bring us closer to finding effective new treatments, and ultimately a cure for all and Alex's Wish has supported 37 projects to date, from early-stage research through to drug development some of which are now showing promising signs in clinic.
- Alex's Wish has funded early-stage research into a promising new steroid alternative drug called Vamorolone, which is showing great promise in clinic and has now been designated Promising Innovative Medicine (PIM) for treatment in Duchenne muscular dystrophy - this is the initial step towards access to the drug on the Early Access to Medicines Scheme (EAMS).
- We have funded five clinical posts though The DMD Hub helping ensure patients in the UK with Duchenne, have access to clinical trials. A clinical trial is often the families only hope of accessing potential new treatments that might one day become approved medicines. When we learnt that clinical trials were being turned away from UK hospitals, because of the lack of doctors, nurses, and physiotherapists to run them, a collaboration of several Duchenne charities collaborated to form the DMD Hub, Alex's Wish being one of them. The DMD Hub has already helped 574 boys get onto clinical trials since 2015. Additionally, 11 UK hospitals are conducting 53 clinical trials into potential treatments for DMD. It was cited as an excellent example of digital

infrastructure in a recent report by the Association of Medical Research Charities.

- Alex's Wish funded a project looking at Carriers of the disease (mothers to boys who are affected) as it is well known that Carriers are at a greater risk of having cardiac problems themselves. This project has delivered great results and will pave the way to providing quicker and easier ways of identifying heart problems early to help save lives of those affected.
- Alex's Wish has helped co-fund two technological advances including The DREAM Wheelchair (a wheelchair specially designed by those living with Duchenne to ensure it meets their needs and will be delivered at an affordable price to them) and The SMART Suit (now called ELEVEX), a lightweight wearable device to help improve upper body function in children and young adults.
- Alex's Wish is investing in projects relating to Care in the Community, ensure every single child across the UK has access to the best possible care despite which postcode they live in.
- Other projects funded include Gene Therapy, Stem Cell Therapy, and anti-fibrotic therapies, amongst many more.
- We are a voice to help speed up access to promising drugs by lobbying governments.
- Alex's Wish continues to raise the profile of Duchenne through media interviews across TV, radio, and press.
- We have been awarded several prestigious awards.

Our Team

- Emma Hallam – Charity Founder and CEO (voluntary role)
- Zoe Edwards – Communications and Operations Manager
- Bev Bailey – Events Manager
- Heather Stone – Administrator (works Tuesday and Thursday)
- Board of Trustees: Alexandra Slack (Chair), Chris Everard (co-Chair) Sandesh Jesrani (Treasurer), Andy Hallam, Emma Hallam, Janine Edwards, Glynis Wright, Sally Barnett, William Hazlerigg & Aishah Iqbal.
- Support from our volunteers and ambassadors.

To find out more, or to support our work, please contact Charity Founder emma@alexswish.co.uk or call 07903 349475.

Thank you.