Help us Conquer Duchenne and save thousands of lives

Alex's Wish is an award-winning Charity based in the heart of the East Midlands, raising vital funds to help conquer an aggressive, fatal muscle wasting disease that affects 1 in every 3,500 boys born.

Every single week, two families will receive the same devastating news as Emma and Andy Hallam – parents to Alex (now 14 years old) that their child has Duchenne. Having learnt that not enough funding is going into conquering Duchenne, Emma and Andy set-up Alex's Wish back in late 2012 with a mission to eradicate Duchenne.

Currently, there is **NO Cure** and **No Treatments** to help slow down the muscle wasting. Alex's Wish only invests in the best science that will help bring about new treatments and ultimately a cure for this generation of children.

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.

What is Duchenne

Duchenne is a genetic disease that causes muscle weakness and wasting. 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.

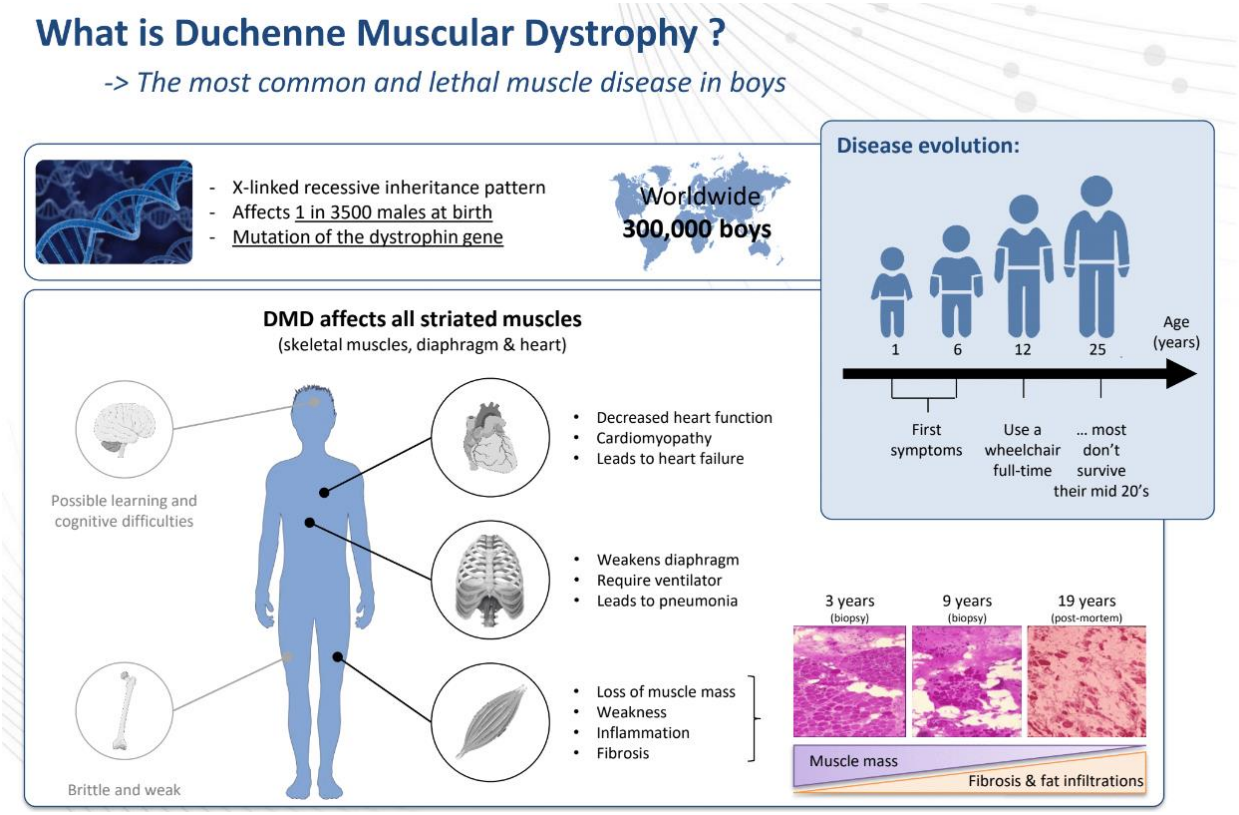
Often the earliest indication that a child may have Duchenne is their difficulty in getting up from the floor, walking or running. Gradually, the child's other muscles will begin to weaken as well. Without dystrophin, muscles become inflamed over time. As the muscles try to repair themselves, fatty and 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 and more young adults with Duchenne lead full lives – going to

university, having relationships and living independently. However, men and women with Duchenne do have a reduced life expectancy.

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, muscle wasting disease – it is relentless – affecting every single muscle in the body. 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. The average age boys live too is just mid 20's mainly 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 die
- No treatments to help all mutations and no cure

Our Mission

Within the next 5 years we want to bring effective new treatments to all those affected and within the next 10 years we want to stop the devastating effects this condition has on children and young adults living with Duchenne. We want to save and improve their lives.

Our Urgent Need for Funding

Duchenne current has a high unmet medical need. We need to provide substantial funding to several clinical trials and research projects over the next 5 years that will provide a catalyst to drive significant investment from biotech and pharmaceutical companies. We are delighted to be able to say that we have now reached our initial target of £1M raised in Autumn 2019, but our work will not stop until a cure is found. We have set ourselves an ambitious goal to raise the next £1M within the next 3 years.

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 it is down to charities like Alex's Wish to do this. It is likely multiple drugs / a combination of therapies will be needed to repair, restore or replace the missing dystrophin protein in Duchenne.

Having met and spoken to some of the best organisations in the world working to conquer Duchenne for the very first time, we believe there is likely to be **a chance of a breakthrough**.

This generation of children could be the ones to survive this condition – but only if action is taken now. Alex is now 14 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

Since launch, we have achieved:

- All money raised has been invested into promising scientific research to bring us closer to finding new treatments, and ultimately a cure for all, we have funded and pledged £700K into 23 projects from early-stage research through to drug development
- We have funded a 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 £150K+ into 5 clinical posts, ensuring we have enough doctors and nurses available in the UK to run the clinical trials specifically for Duchenne
- We have funded a further £10K into supporting boys with Duchenne in Birmingham Heartlands Hospital
- We have a voice to help speed up access to promising drugs by lobbying governments
- We have raised the profile of Duchenne across the East Midlands through media interviews across TV, radio and press and appeared on BBC East Midlands Today
- We have been awarded several prestigious awards including:
    - Volunteer of the Year 2019 was awarded to Emma Hallam by the Voluntary Awards Leicester & Leicestershire (VALL)
    - High Sherriff of Leicestershire Tim Maxted awarded Alex's Wish for Outstanding Contribution in the Local Community 2018

- Enterprising Women's Awards 2017 – Alex's wish was award with Highly Commended for the Community Champion of the Year category
- Leicester Mercury Business Awards 2016 awarded Alex's Wish as the winner of the Non-for-Profit category
- Forward Ladies Women in Business Awards 2016 awarded Alex's Wish as the winner of the Not for Profit category
- A special award was given by the Institute of Directors East Midlands to Emma Hallam for Personal Impact - Ron Lynch, East Midlands Regional Director quoted "We were wowed by Emma's enthusiasm and passion for the cause."

What Projects We Fund

We are currently funding various options, including:

- Dystrophin restoration
- Respiratory and cardiac treatments
- Muscle growth and regeneration
- Inflammation and fibrosis
- Doctors and nurses to help run clinical trials in the UK

We are a Partner Charity of **Duchenne UK** – a lean, ambitious charity dedicated to conquering Duchenne Muscular Dystrophy. By partnering with Duchenne UK, we have more chance of reaching our goal. We understand that collaborating with others is vital if we are to conquer this disease.

We work with their 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 within the next 5 years.

Alex's Wish has also formed a partnership with the **Muscular Dystrophy UK** and 5 other charities dedicated to beating Duchenne. The collaboration is called the **Duchenne Forum** with a prime purpose of accelerating progress in the search for effective treatments and eventually cures.

www.alexswish.co.uk

Watch our short video: **https://www.youtube.com/watch?v=VqQWsQUyNiw**

See Alex in the World's Strongest Boy commercial: **https://youtu.be/HAlSE_nkurU**

Facebook: **www.facebook.com/alexswishcharity1**

Twitter: **www.twitter.com/alexswish**

Our Team

- Emma Hallam – Charity Founder, CEO and mum to Alex (voluntary)
- Zoe Edwards - Events & Fundraising Manager (full-time)
- Heidi Eastell - Events & Fundraising Coordinator (part-time)
- Our growing Board of Trustees: Stephen Dean (Chair), Sandesh Mukesh (Treasurer), Andy Hallam, Emma Hallam, Chris Everard, Janine Edwards, Glynis Wright, Rachel Hargrave, Sally Barnett and Alexandra Slack