



Accelerating the CURE.
Redefining CARE.
Building COMMUNITY.

# Duchenne is the most severe form of muscular dystrophy. It is 100% fatal.

Duchenne affects one in 5,000 male births. It primarily affects males, but can also affect females. In all cases, Duchenne is caused by mutations in the DMD gene.

# Duchenne's impact on the body:



300,000

Children & Young Adults Worldwide Living with Duchenne



5

Typical Age of Diagnosis



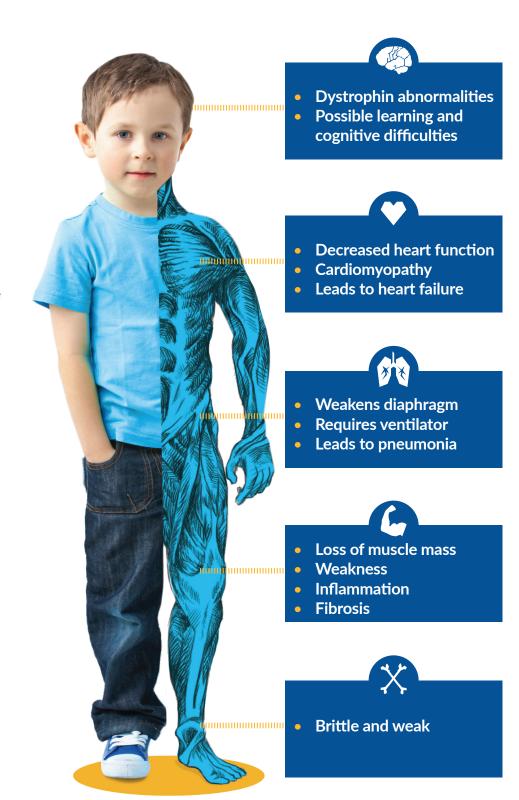
**12** 

Most Lose Ability To Walk Before Teenage Years



**20** 

Most Don't Survive Beyond their Mid-20s



# Who We Are

With a mission to cure Duchenne muscular dystrophy, CureDuchenne breaks the traditional charitable mold and balances passion with business acumen. Our innovative venture philanthropy model funds groundbreaking research, early diagnosis and treatment access. With pioneering education and support programs, our organization drives real change for those with Duchenne muscular dystrophy and their loved ones.

# **CureDuchenne's Impact Since 2003**

- Raised over \$50M for research and to develop impactful programs that help Duchenne families
- Co-founded Exonics, the first gene-editing company for Duchenne
- Sponsored 12 projects that have progressed to human clinical trials
- Provided early funding for research into the first FDA-approved Duchenne drug
- Launched regional education events for underserved patient populations
- Pioneered the only Duchenne physical therapy certification program







#### Investment

## **Impact**

2019

CureDuchenne in Collaboration with AskBio invests in Touchlight AAV



2019

Improving large scale gene therapy manufacturing



2017

CureDuchenne provides seed funding and co-founds Exonics Therapeutics for CRISPR Cas9 research



**UTSouthwestern** 

Medical Center.

2019

Vertex acquires Exonics to develop the preclinical program



2016

CureDuchenne funds Bamboo Therapeutics





2019

Pfizer acquires Bamboo and begins gene therapy clinical trials



2010 - 2017

CureDuchenne funds Nationwide Children's U7 AAV delivered exon skipping Duplication 2 research





2019

Audentes partners with Nationwide for Duplication 2 clinical trial and permanent exon skipping of exons 51 and 53



2010

CureDuchenne awards grant to Sarepta Therapeutics





2016

Exondys51, first drug approved for Duchenne



**2018** 

Gene therapy trial shows significant dystrophin increase and clinical benefit



Supports research across the globe by leveraging venture philanthropy to encourage new drug treatment, funding and fostering promising early-stage science to find a cure for Duchenne.



We have partnered with RUCDR Infinite Biologics and UC Irvine, to create the CureDuchenne Biobank collecting minimally invasive blood and skin tissue samples that will be banked and made openly accessible for research.



An interactive education and outreach program designed to provide the community with information, resources and best practices for managing the challenges of Duchenne and improve overall quality of life. The CureDuchenne Cares program is fully funded by sponsorship partners and offered at no charge to the community.



The CureDuchenne Physical Therapy certification is a designation held by physical therapists who have completed advanced training in Duchenne and have accepted the responsibility to provide an excellent standard of care to those with Duchenne. The physical therapists benefit from on-going education from CureDuchenne and patient referrals. The Duchenne community benefits from being able to identify physical therapists who meet the standards of care that they can trust.



The DuchenneXchange is a free, collaborative online platform for patients and families and advocacy organizations to connect and share knowledge. Built by the Duchenne community for the Duchenne Community.

### **Contact Us**

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