



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



- Dystrophin abnormalities
- Possible learning and cognitive difficulties



- Decreased heart function
- Cardiomyopathy
- Leads to heart failure



- Weakens diaphragm
- Requires ventilator
- Leads to pneumonia



- Loss of muscle mass
- Weakness
- Inflammation
- Fibrosis



- Brittle and weak

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

askbio

2017

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



2019

Vertex acquires Exonics to develop the preclinical program

exonics
THERAPEUTICS

UT Southwestern
Medical Center

VERTEX

2016

CureDuchenne funds Bamboo Therapeutics



2019

Pfizer acquires Bamboo and begins gene therapy clinical trials

Bamboo
therapeutics

Pfizer

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

NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.

AUDENTES

2010

CureDuchenne awards grant to Sarepta Therapeutics



2016

Exondys51, first drug approved for Duchenne

SAREPTA
THERAPEUTICS

EXONDYS 51
(eteplirsen) Injection

2018

Gene therapy trial shows significant dystrophin increase and clinical benefit

Help us continue to fund a cure.
Donate today at CureDuchenne.org/Donate



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

CureDuchenne.org | 949.872.2552 | info@cureduchenne.org
1400 Quail Street, Suite 110, Newport Beach, CA 92660

Federal 501 (c) (3) Tax ID: 20-0299958