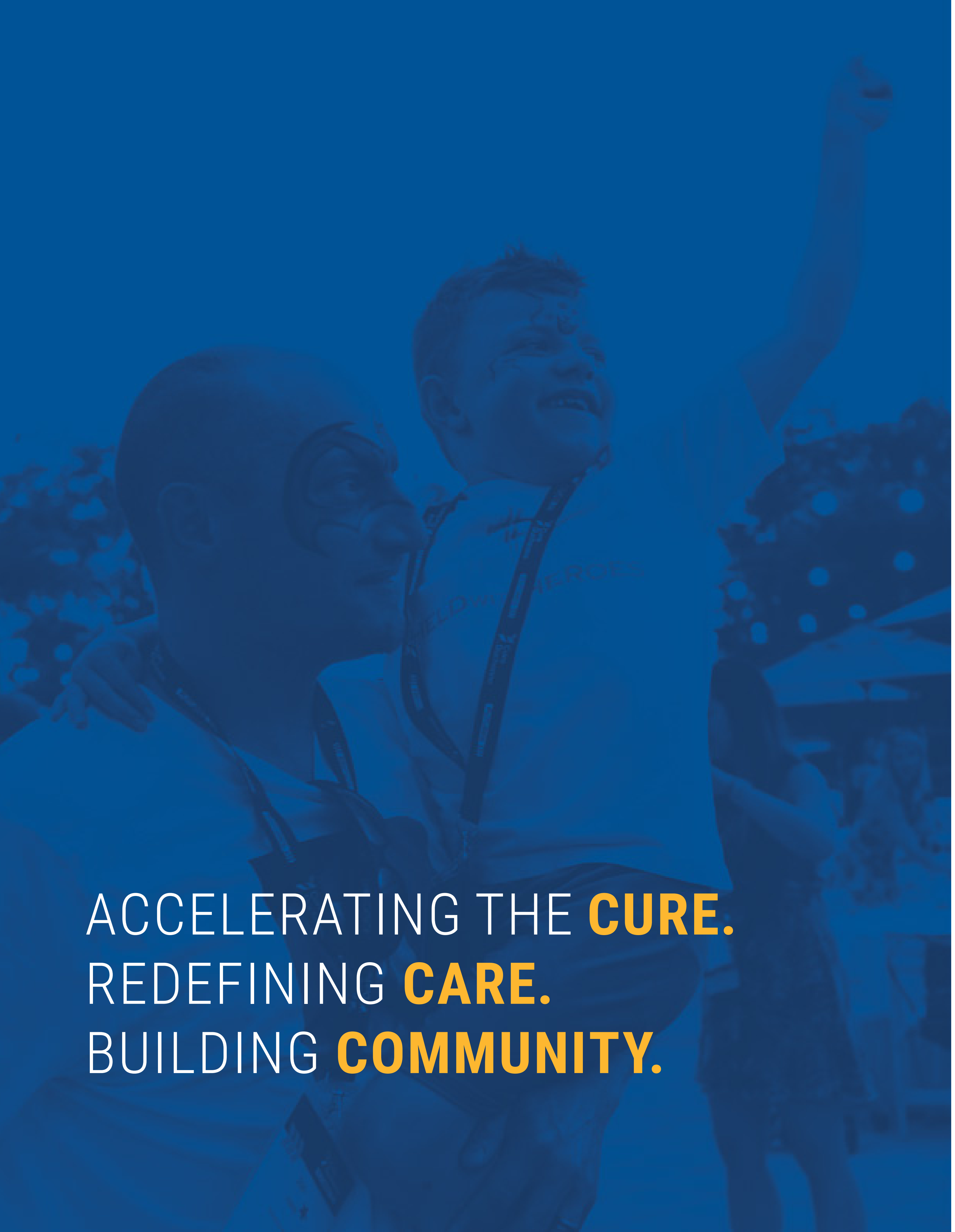
Cure
Duchenne®

Cure. Care. Community.



ACCELERATING THE **CURE.**
REDEFINING **CARE.**
BUILDING **COMMUNITY.**

A MESSAGE FROM **OUR FOUNDERS**

Thank you to our donors and community for your ongoing support of CureDuchenne. Your generosity since our founding nearly 20 years ago has made a difference in the lives of 300,000 children and young adults affected by Duchenne muscular dystrophy worldwide. By supporting CureDuchenne, you are helping add years to the lives—and life to the years—of these boys. We are grateful for the successes achieved and the hope we have provided.

When our son Hawken was diagnosed with Duchenne almost 20 years ago, our physician didn't expect him to live beyond his late teens. Faced with a seemingly insurmountable situation, we founded CureDuchenne, an organization with the sole mission of finding and funding a cure for this devastating disease. With your help, we've made great strides in our efforts to discover promising science and improve support resources that families, caregivers, and physical therapists need to navigate the challenges of this progressive disease.

With your support, CureDuchenne directs nearly 90% of our proceeds to research and mission-critical services. Together, we celebrated the first FDA approval of a treatment for Duchenne in 2016, embarked on an ambitious research partnership in gene-editing technology, and brought 16 potential treatments to human clinical trials. Since our inception, the quality of life and expectancy for those living with Duchenne has increased significantly, but this is still a race against time.

Everyone affected by Duchenne is counting on the miracle of your support. Your compassion and goodwill are what enables CureDuchenne to sustain a pipeline of transformative research and outreach programs to help give thousands worldwide real hope for a cure. Together, we can CureDuchenne.

Sincerely,

Paul, Debra & Hawken

PAUL, DEBRA, AND HAWKEN MILLER

What Is Duchenne Muscular Dystrophy?

Duchenne muscular dystrophy is a degenerative neuromuscular disease that is 100% fatal. It is the most common and severe form of muscular dystrophy, affecting roughly 1 in 5,000 male births.

Duchenne is caused by mutations in the gene that encodes dystrophin, a key muscle protein that plays a critical role in maintaining muscle health. Without dystrophin, cells become damaged and die easily, resulting in heart and breathing failure.

Children with Duchenne have significant delays reaching early development milestones, and most will require the use of a wheelchair by ages 10–12. In late teens, their heart and breathing muscles are severely impacted, and many Duchenne patients do not survive beyond their late 20s. The disorder knows no cultural, economic, or social boundaries.

DUCHENNE BY THE NUMBERS

300,000

Number of individuals living with Duchenne worldwide

5

Typical age of diagnosis

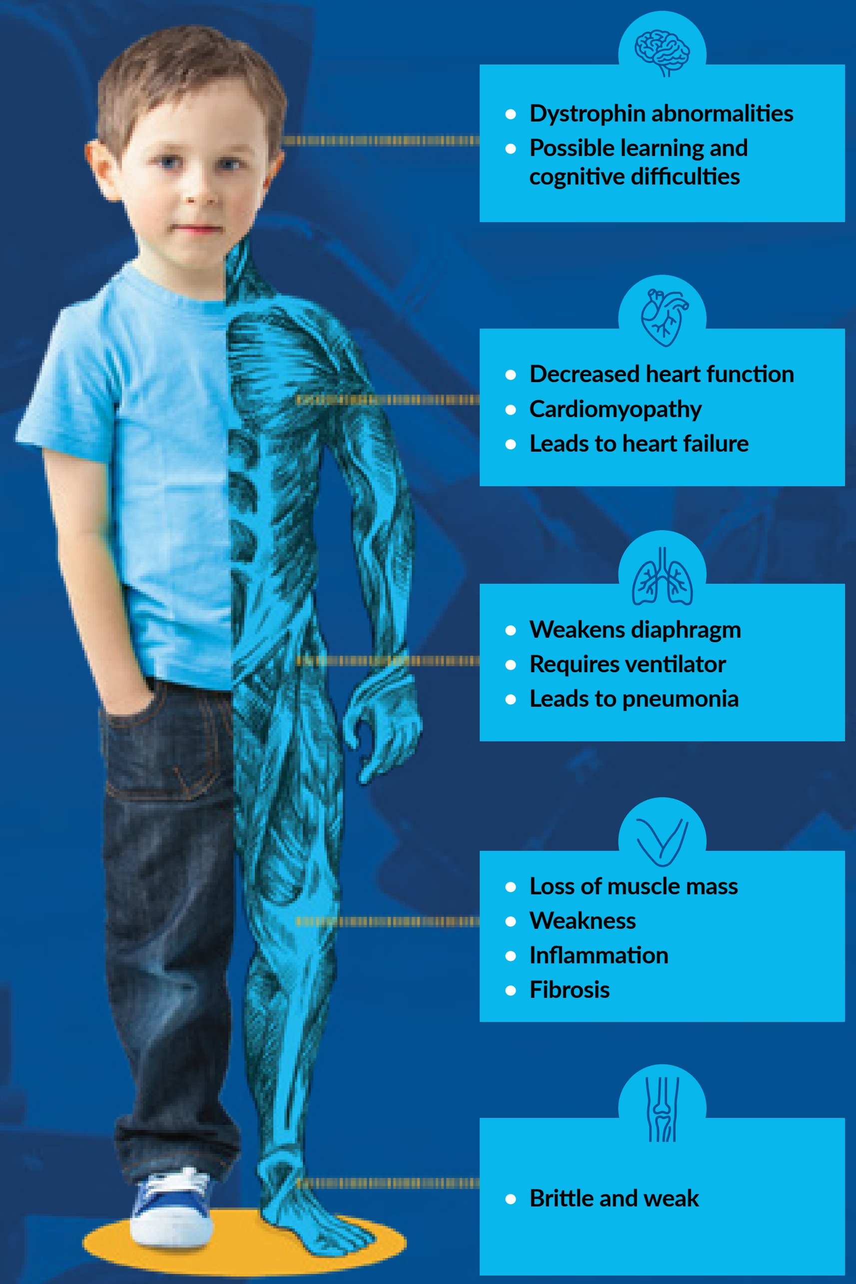
12

Most lose the ability to walk before their teenage years

20

Most don’t survive beyond their mid-20s

HOW DUCHENNE AFFECTS THE BODY



- 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**

Our vision is our name: **to cure Duchenne.**

We are committed to improving the lives of everyone affected by Duchenne by accelerating research to find the cure, improving care, and empowering the Duchenne community.

The CureDuchenne staff at the 2021 Futures conference in Orlando.

2021–2022 **Board of Directors**

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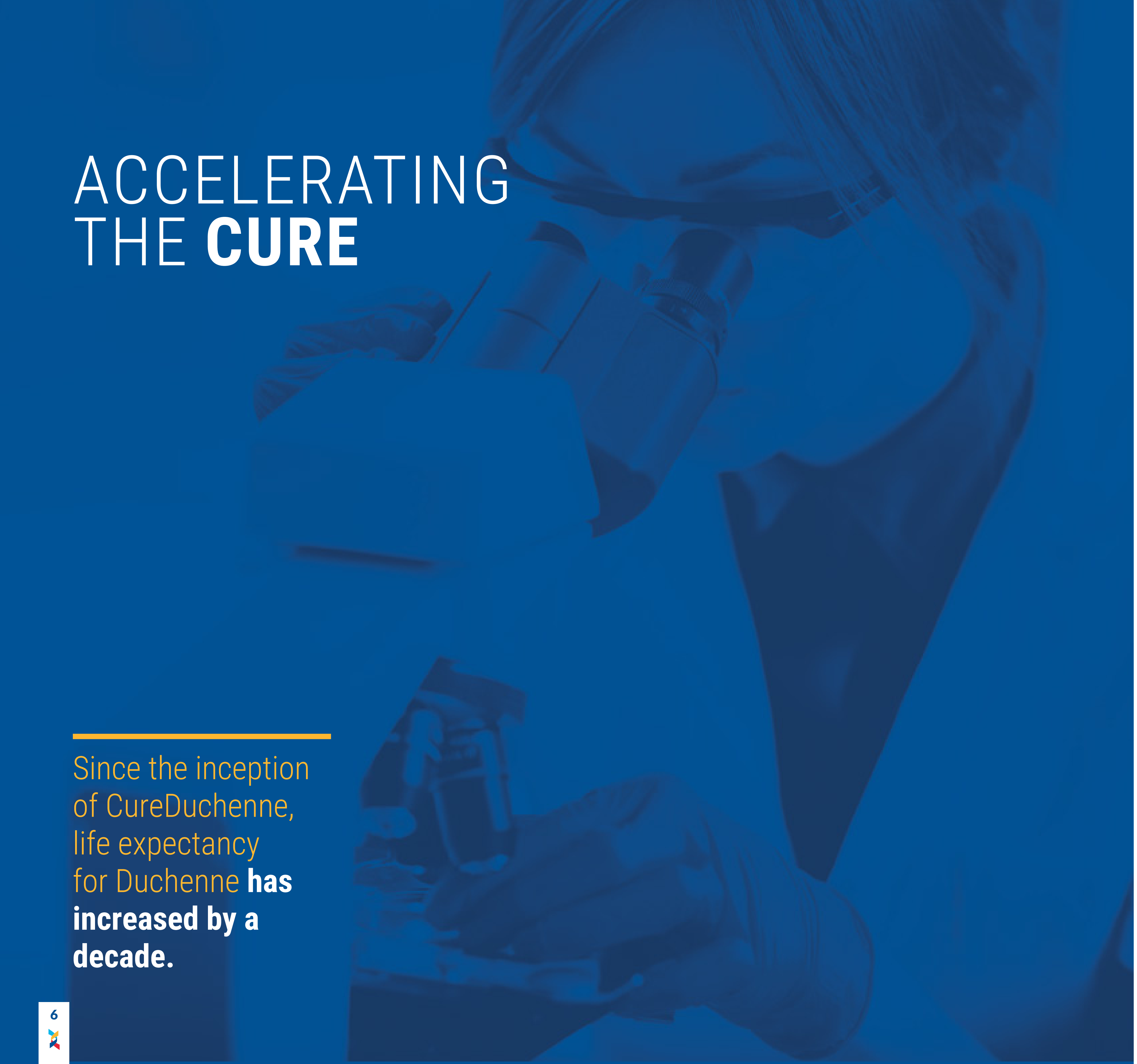


ACCELERATING THE **CURE**

Since the inception of CureDuchenne, life expectancy for Duchenne **has increased by a decade.**

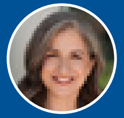
CureDuchenne is the leading nonprofit funding Duchenne research. We focus on high-impact research addressing critical gaps and unmet needs in treatment and deploy in-house expertise to guide and support projects for success. We take a holistic approach by funding research from early startup through clinical development.

Venture Philanthropy: A Model of Innovation

We find, fund, and foster early-stage science. Through an impact financing model, CureDuchenne Ventures supports Duchenne research by redeploying returns made on previous investments to support new drug development opportunities. Our robust portfolio of companies and projects we support covers a diverse array of state-of-the-art technologies aimed at combating Duchenne.

OUR EXPERT SCIENTISTS

Michael G. Kelly, PhD
Chief Scientific Officer



Lianna R. Orlando, PhD
Vice President of Research

Find

Find the emerging technologies and research with the most promise for treating Duchenne

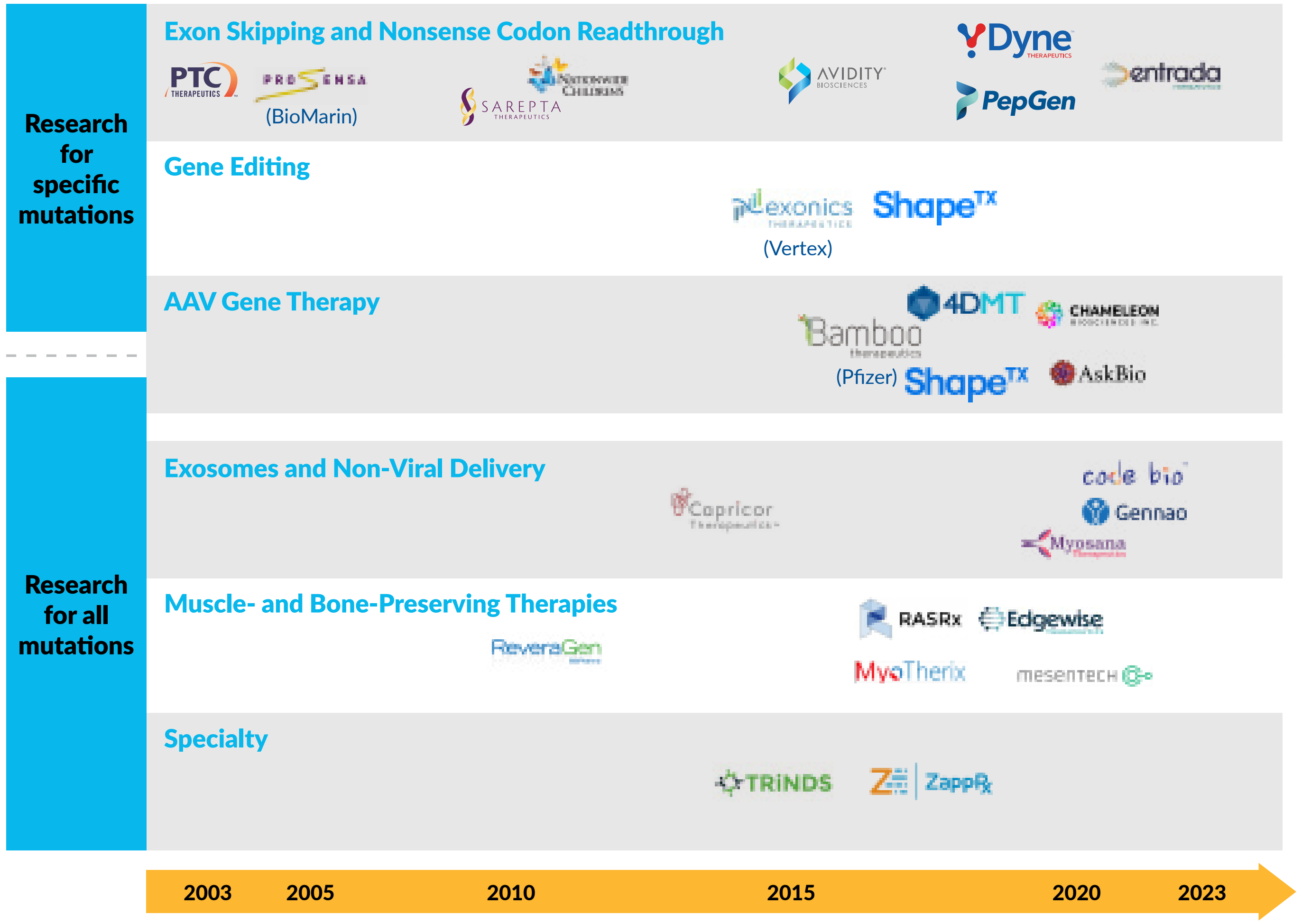
Fund

Fund research programs that address gaps and key unmet needs within the current clinical pipeline

Foster

Foster long-term strategic relationships with our biotech and pharma partners

Pioneering a Pathway to a Cure



Drug discovery to FDA approval can take up to 10 years and cost $2.6 billion.

CureDuchenne Ventures strategically guides promising research at early and critical stages, and accelerates the development process.

Funding Groundbreaking Research and Treatments

CureDuchenne accelerates research by identifying and investing wisely in encouraging research, applying expert due diligence to reduce the risks embedded in early-stage science, and helping accelerate the most promising new treatments for Duchenne muscular dystrophy.

> Without CureDuchenne, we would not be where we are today—they enabled our progress and our success. Beyond just their financing, we depended on their business and financial experience and their connections in the Duchenne community, which gave us the credibility we needed.
>
> **Eric Olson, PhD, Professor and Chair of Molecular Biology at UT Southwestern and co-founder of Exonics**

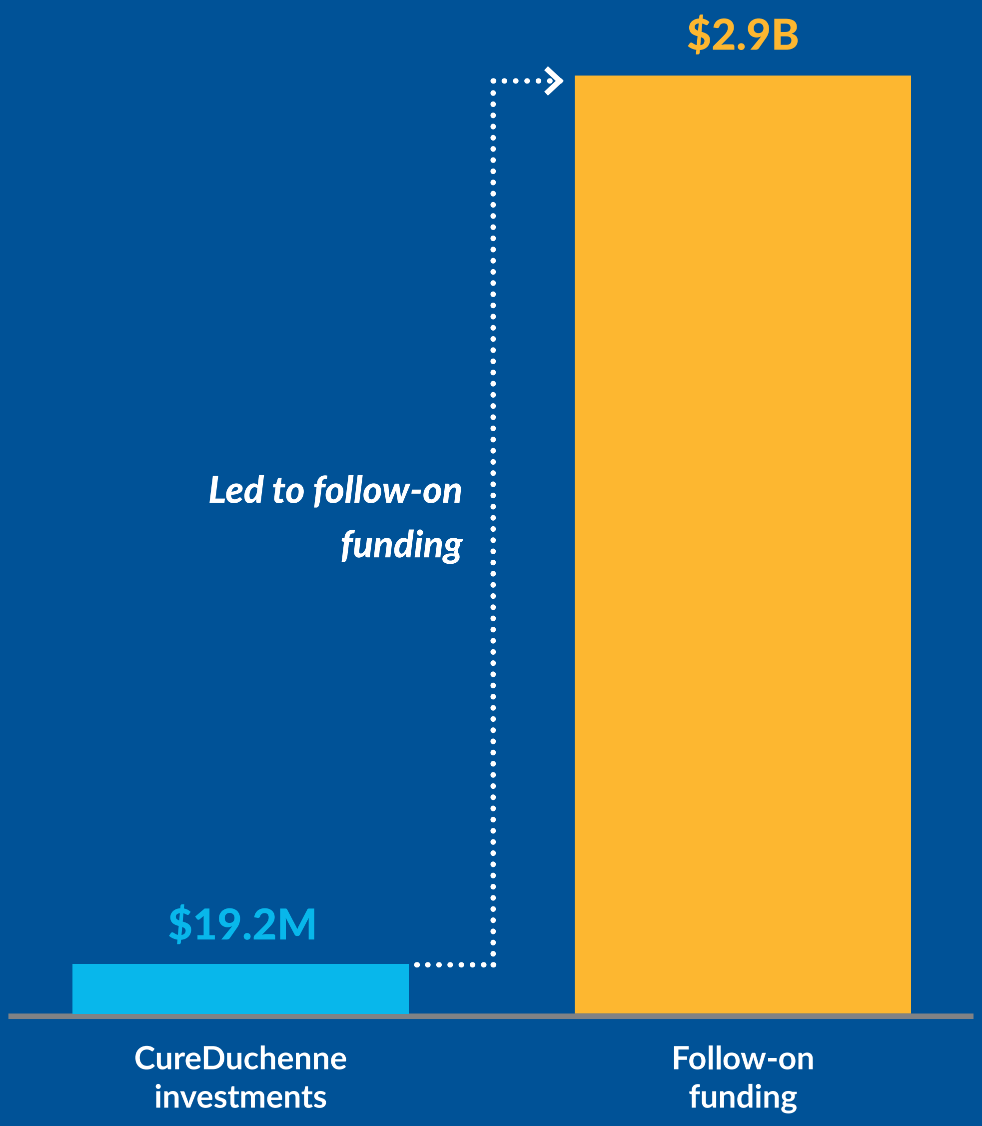
SINCE INCEPTION, CURE DUCHENNE HAS:

- Contributed early funding to the first FDA-approved Duchenne drug
- Funded 44 research projects, including investments in 24 companies
- Funded 16 research projects that have advanced to human clinical trials, with more starting soon
- Invested more than $26M toward research and research-related activities, including a biobank and newborn-screening program
- Leveraged nearly $3B in follow-on investments from venture capital, biotech, and pharmaceutical companies to advance Duchenne therapies
- Reinvested 100% of investment returns into research, resources, and support for families living with Duchenne

Where We Go, Funding Follows

CureDuchenne has invested more than $26M in research through grants and investments. Of that $26M, $19.2M in biotech investments has led to follow-on funding of nearly $3B.

CureDuchenne has a track record of successful investments, and its "stamp of approval" attracts future investments in these projects from VC firms, biotech companies, and pharmaceutical companies.



Newborn Screening

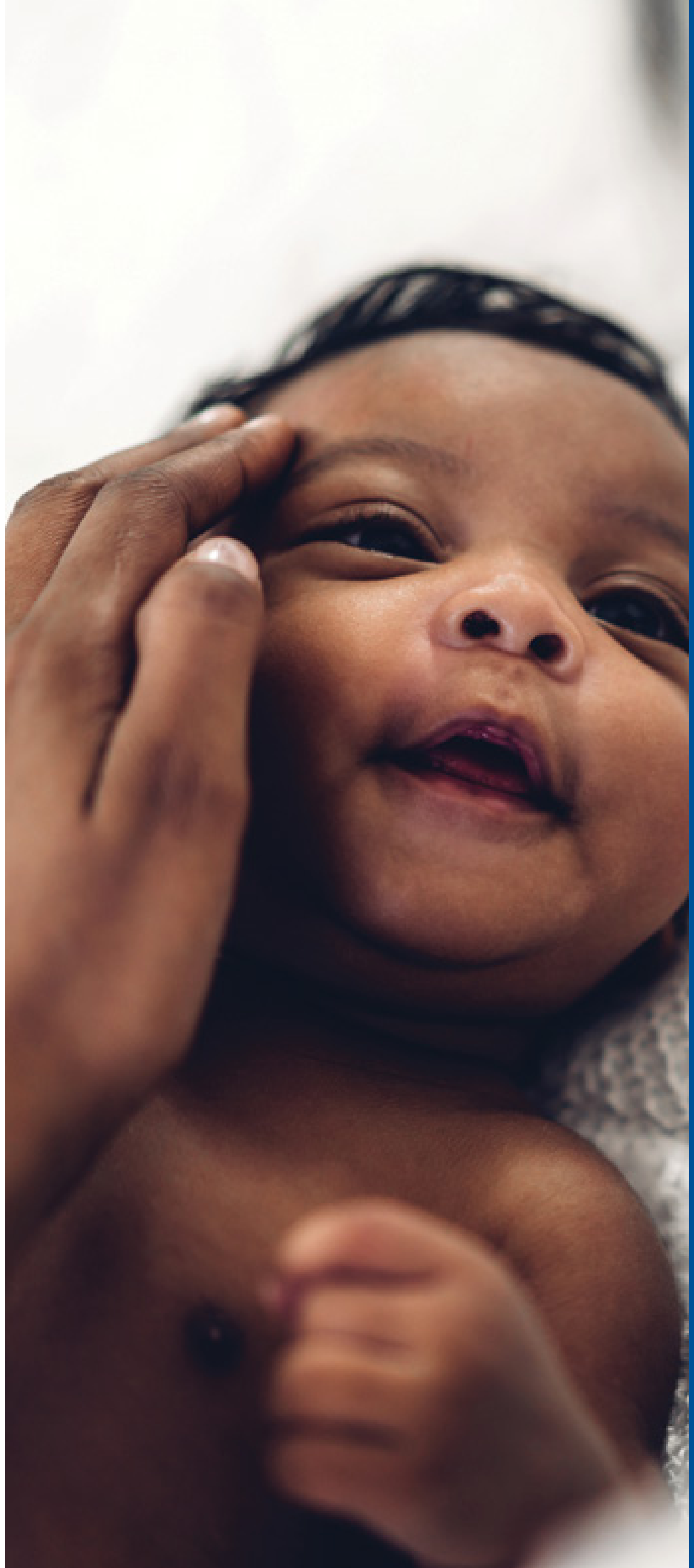
Newborn screening (NBS) is critical in identifying individuals with Duchenne earlier so that care can begin immediately when treatments could be most effective. Currently, diagnosis occurs after the observance of symptoms, and, therefore, therapy may not begin until age 5.

In June 2022, CureDuchenne was involved in nominating Duchenne muscular dystrophy to be added to the Recommended Uniform Screening Panel (RUSP), a list of disorders that are screened for at birth. A CureDuchenne-funded pilot NBS program at Brigham and Women's Hospital in Boston, along with several pilot NBS programs, contributed data to this effort.

CureDuchenne continues to fund the program at Brigham and Women's Hospital, which has screened more than 6,400 babies to date, with hopes to expand the program throughout the state.

6,483

Number of babies screened for Duchenne at Brigham and Women's Hospital



Linking the Community to a Cure

CureDuchenne Link is a data-integrated biobank that provides researchers with vital data and biosamples from individuals with Duchenne and Becker muscular dystrophy as well as carriers. Open to any mutation, age, or mobility status, this robust and longitudinal agnostic repository is available to qualified researchers (academic and pharma). Breaking the mold of siloed projects, CureDuchenne Link allows researchers access to coded data and biosamples in one all-encompassing platform.

CureDuchenne Link goes beyond any registry or biobank. It provides vital resources to accelerate discovery and bring treatments faster, making contributions more meaningful than ever.

THE POWER OF A BIOBANK

> Biobanks like CureDuchenne Link are well-established, well-regulated, and the ethically proper way to set the stage for studies.
>
> **Kevin Eggan, PhD, Group Vice President, Head of Research and Early Development, BioMarin Pharmaceutical, Inc.**

REDEFINING CARE

We help families navigate the Duchenne journey with resources and expert support **they can rely on.**

As we actively pursue transformative treatments, CureDuchenne understands the needs of the Duchenne community.

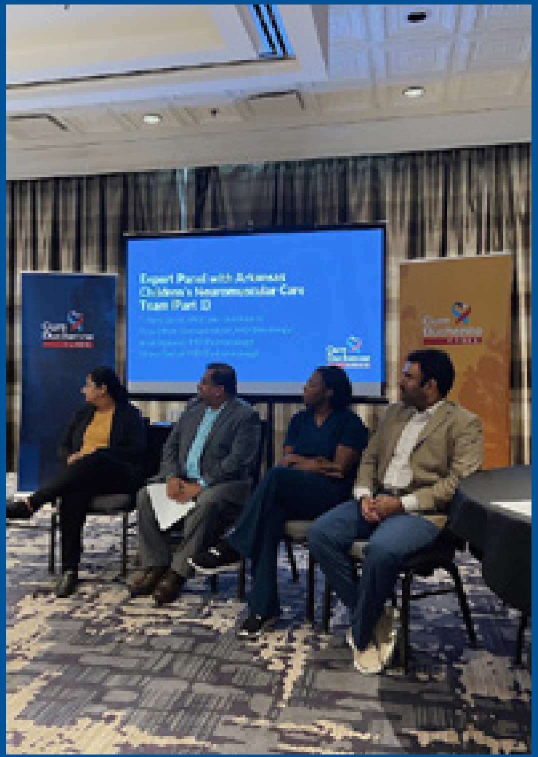
Our network of in-house healthcare experts and industrywide collaborators are current on all treatment options, from physical therapy to therapeutics and clinical trials. These experts counsel and educate Duchenne patients and families on the best care options and bring the Duchenne medical community together to improve standards of care.

Delivering the Best Care

CureDuchenne Cares is an interactive education and outreach program for parents, caregivers, physical therapists, and allied health professionals who serve patients living with Duchenne. We fulfill an unmet need for information, resources, and best practices for managing the challenges of Duchenne to help improve a patient's quality of life.

SNAPSHOT OF SERVICES

- **Sessions:** Intimate dinner events for caregivers to provide connectivity in local communities
- **Workshops:** Full-day educational workshops covering the latest in care and research
- **CureDuchenne 1:1:** Individualized, unbiased information and advice from experts
- **Medical ID Bracelets:** Wristband that communicates important information about Duchenne to EMS professionals in urgent medical situations
- **CureDuchenne Advocacy:** Champion causes and influence policies by sponsoring legislation and educating key decision-makers
- **Virtual Events**: Educational webinars and social events that reach families around the globe
- **Clinic Busy Bags**: Information and resources for families as well as engaging activities for kids to keep them engaged for long hours at clinics



CARES AT A GLANCE

27 Cares events
2021–2022

10,000+ individuals impacted
since inception

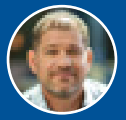
> When our son was diagnosed with Duchenne, we had no idea where to start looking for the best doctors and care. I reached out to CureDuchenne, whose experts talked with us and gave us resources and a personalized checklist to make sure he was getting proper care. I don't know what we would have done without CureDuchenne.
>
> **Catalina Rodriguez, mom**

The First Name in Professional Training

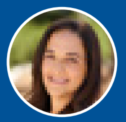
The CureDuchenne Physical Therapy and Professional Program is the first physical therapy education program specifically designed to help individuals living with Duchenne prolong ambulation and delay many areas of disease progression. It also is the only program that offers a physical therapy certification for treating people with Duchenne.

The Physical Therapy and Professional Program is consistently at the forefront of the field, evolving new methods and techniques to provide optimal care and training therapists in distant corners of the world with online and in-person professional training.

OUR EXPERT TEAM



Doug Levine, PT
Physical Therapist



Jennifer Wallace Valdes, PT
Physical Therapist

PT AT A GLANCE

109 physical therapists educated
2021–2022

1,500 physical therapy course attendees
since inception

> The CureDuchenne Cares program is one of the most impactful courses I've ever taken. This program has empowered me to become the best physical therapist that my Duchenne patients and their families need.
>
> **Dani Trees, PT, Texas**

Our Global Reach

Our team trains allied healthcare professionals around the world, helping people maintain mobility, flexibility, and quality of life at all ages and stages of their journey with Duchenne. Below is a list of countries we have provided training in:

Argentina
Canada
Colombia
England
Hungary
India
Italy
Mexico
Nigeria
Russia
South Africa
Uganda
United States

> Working with Duchenne patients is different than other patients you work with as a PT. What is normal for other patients could be detrimental to a Duchenne patient's health.
>
> **Elina Gonzalez, PT, California**

BUILDING COMMUNITY

Until we find a cure, we create connections that **fuel hope.**

CureDuchenne unites the Duchenne community by empowering them to live joyful and meaningful lives while we accelerate treatments and better care.

CureDuchenne conferences, fundraisers, and other events engage families and donors in a true community that offers support and hope.

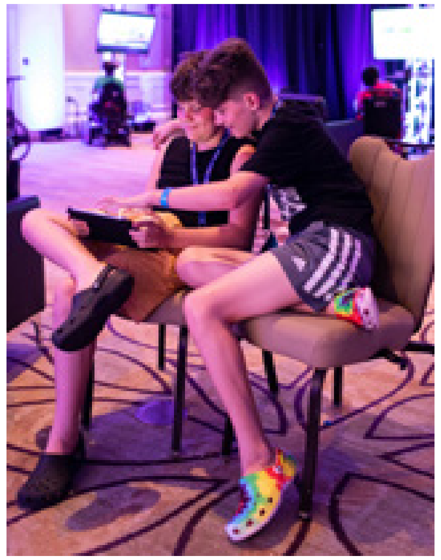
FUTURES National Conference

The CureDuchenne FUTURES National Conference is a hybrid event focused on bringing education, resources, and connection to the Duchenne community in a family-friendly environment.

Whether enjoyed virtually or in person, FUTURES attendees received exclusive access to important updates on therapeutic research, thoughtful discussions on the emerging approaches to care that enhance quality of life, a wealth of valuable resources, and time well-spent with the Duchenne community.

This annual event offers insightful and interactive presentations on a variety of relevant topics, an exhibitor showcase, social events for the whole family, e-Gaming experiences, and more.

> The CureDuchenne FUTURES Conference was amazing, inspiring, and full of hope. Being with other people going through the same rare disease allows you to feel not so rare, and that is life changing.
>
> **Vanessa Riley, mom**



FUTURES BY THE NUMBERS

1,600

Attendees hosted since 2019

260

Sponsors, exhibitors, and speakers since 2019

The Napa Wine Series

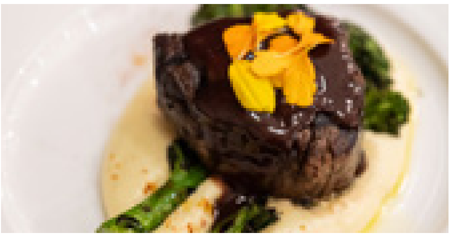
Our signature Napa Wine Series is a celebrated fundraising forum for donors and supporters. Every year, we introduce this community to renowned vintners and wine enthusiasts, inviting them to taste the best of wine country. Attendees sip coveted wines during a grand tasting and enjoy a perfectly paired multi-course meal at vintner-hosted tables before bidding on extraordinary auction lots.

This series drives our fundraising efforts across the country, with events organized in Newport Beach, California, Miami, and Philadelphia.

CUMULATIVE RAISED:

$10.2 million

> Science is getting closer to finding a cure for this devastating disease that impacts my son and more than 300,000 other amazing boys worldwide, and fundraisers like Napa in Philadelphia are so critical to our efforts. We remain relentless in this fight, and it's wonderful to see so many people support our mission to find a cure for this disease that so few have heard about.
>
> **— Manu Gambhir, father and event co-chair**



> Duchenne is a devastating disease that cuts short the lives of thousands worldwide. Paige and I joined the cause to help find a cure 11 years ago, but we never imagined what this tournament could become. CureDuchenne has made a tremendous impact, investing into promising science that moves the needle closer and closer to a cure every day, and I'm proud of what we've accomplished together over the past decade.
>
> **Ryan Getzlaf, NHL All-Star and retired Anaheim Ducks captain**

The Getzlaf Golf Shootout

#1 GOLF TOURNAMENT IN ORANGE COUNTY

With the power of expanding our community of those united in pursuit of a cure, the Annual Getzlaf Golf Shootout brings together professional athletes, celebrities, and community leaders in support of CureDuchenne. Participation in the Getzlaf Golf Shootout is an opportunity for businesses and sports fans to exercise social responsibility through an event that is both entertaining and impactful.

The Annual Getzlaf Golf Shootout takes place at the Monarch Beach Golf Links in Dana Point, California.

CUMULATIVE RAISED:

$5 million

The Families That Champion the Mission

Attending, hosting, or sponsoring a CureDuchenne fundraising event helps raise critical funds to save lives. From galas to golf tournaments, we work with families to develop and host events across the country. Through the generosity of passionate and generous donors, event sponsors, media partners, and families, we will cure Duchenne!

CUMULATIVE RAISED:

$8.8 million


1


2


3


4


5


6


7

1 Paul and Laura Heaton, Calves to Cure DMD. 2 Ryan and Paige Getzlaf, Getzlaf Golf Shootout. 3 Brad and Misty Foster, Dinking for Duchenne. 4 Manu and Sonal Gambhir, Napa in Philadelphia. 5 Chris and Susan Finazzo, Napa in Miami. 6 Tim and Laura Revell, Champions to CureDuchenne. 7 Ramiro and Maribel Munoz, Dealing for Duchenne.

Contact Us:

CureDuchenne.org
949.872.2552
info@cureduchenne.org

100 Bayview Cir, Suite 5600
Newport Beach, CA 92660

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