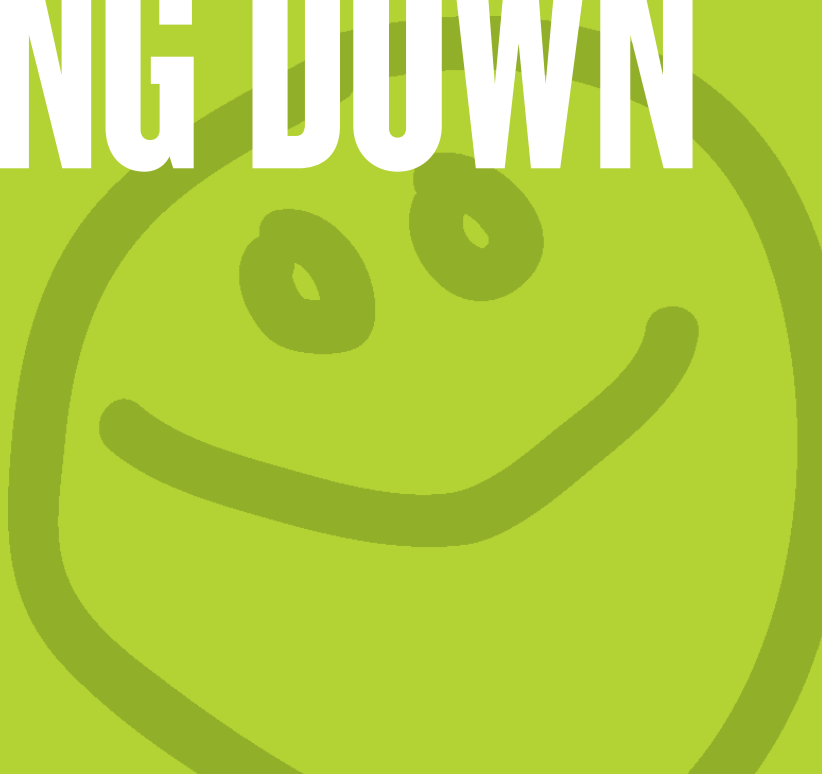
CHARLEY'S FUND 2015:

DIGGING DEEP + DOUBLING DOWN



DEAR FRIENDS AND SUPPORTERS

From the day Charley was diagnosed we have had one clear goal: to accelerate the development of life-saving treatments for Duchenne muscular dystrophy. For a decade we have remained focused like a laser on that singular mission, and we are now starting to see some tangible results.

This year's annual newsletter features photos of children who are experiencing real-life benefits, thanks to your support. A teenager who can cut his own meat for the first time in years. A child who can now climb into the family SUV without assistance. A young man who has needed help with almost every daily task now brushes his teeth by himself. You have made these profound victories possible.

The road to new therapies is by no means linear. Along the way we have faced — and will continue to face — complex problems. When we launched Charley's Fund in 2004, the most urgent need was financial support to help advance promising research. That is still the case, but over the years it has become clear that in many instances, it's not only money that's missing. Critical thinking and creative solutions are often the catalysts needed to advance promising research that is stuck or moving too slowly. So on these pages we also share examples of how we are constantly evolving to solve these problems.

Your support has given birth to our historic progress, so the photos you see on these pages are your pride and joy. Speaking of which, our own middle one continues to wring every possible drop of fun and laughter out of life. This past year, Charley partied with Snoop Dogg at the Matt's Promise benefit, rocked to Rihanna at the Barclay's Center, and cooked live lobsters on summer vacation.

Now 15 years old, Charley is talking about driver's ed and college credits, topics that trigger extreme mixed emotions. These very real reminders that time is slipping by too quickly motivate us to double down and work even harder to make sure new medicines are developed as quickly as possible. At the same time, we are filled with gratitude that you have enabled our son and other children like him to not only dream about — but actually plan — a future the experts did not expect them to have. As Charley gets older and the stakes get higher, we absolutely will not let up the pace until that new future becomes a reality.

With gratitude,

Tracy & Benjy

Tracy and Benjy

(back) Benjy, Tracy, Sam, (front) Maisy and Charley on vacation

CHARLEY IN 2015

Charley and a friend at the US Open.

Catch of the day for Charley and Nate.

Charley chilling poolside.

Snoop helped Charley celebrate 15 years!

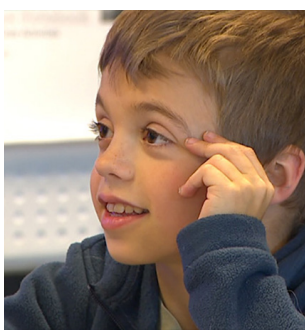
Graduation day with friends.

Fresh lobster dinner!

SEEING IS BELIEVING

YOUR SUPPORT + OUR INNOVATIONS = REAL LIFE BENEFITS

Our progress is incredible. It is historic. But what's truly amazing about what we are accomplishing together is that we're giving children and families the small things that make life worth living.



Aidan is being treated with eteplirsen, an exon skipping drug that could be FDA approved by February 2016. After a year on drug, he can now climb into the family car without help and kick a soccer ball in the back-yard for hours at a time.

Nash is taking HT-100, a pill that reduces scar tissue and enables healthy muscle to grow. The drug was stalled until Charley's Fund knocked down the hurdles standing in its way. One-year data from the clinical trial shows not just stabilization but actual, durable improve-ment in muscle strength.

"Yesterday, June 8, 2015, was a big day in our family," wrote Pietro's parents earlier this year. "Pietro received his first infusion of eteplirsen, a clinical trial treatment." Because Pietro started on the medicine at such a young age, the benefits could be even greater than the improvements older boys are experienc-ing. Already he is able to do things he could not do before, like walk up stairs without holding on.

"Nathan's been cutting his own meat for the first time in years," reports his mother. Nathan's physical therapist also noticed that he's getting stronger. The data from the HT-100 trial certainly show an increase in muscle strength, but it's these real-life improvements that put a smile on our faces.

RESEARCH RUNDOWN 2015

HERE'S A BRIEF TASTE OF SOME RESEARCH HIGHLIGHTS, WITH MORE BIG NEWS IN THE PAGES TO FOLLOW

FOLLISTATIN is a gene therapy approach being developed by Milo Biotechnology at Nationwide Children's Hospital. The therapy, delivered by intramuscular injection, is designed to maintain or restore muscle function in boys affected with DMD. It is based on adeno-associated virus delivery of follistatin 344 to increase muscle strength and prevent muscle wasting and fibrosis. Because follistatin's mechanism of action is not mutation specific, it could potentially help other forms of muscular dystrophy. Two boys have been dosed so far with this experimental treatment.

SMT C1100 is a novel drug candidate that aims to upregulate utrophin. In a recent clinical trial conducted by Summit, ten out of 12 kids who took the medicine achieved plasma concentration levels expected to increase utrophin by approximately 30% or greater. The company plans to initiate a Phase 2 trial with up to 40 patients before the end of this year. They'll use MRI of the leg muscles, muscle biopsies, and functional measures such as the six-minute walk test and the North Star Ambulatory Assessment to determine what effect the drug has in kids with Duchenne.

AT-300 We've all been taught that calcium is good for you, but in Duchenne calcium doesn't always have positive connotations. When a child does not produce dystrophin, a cascade of negative events ensues, one of which is a high level of calcium infiltrating the cells. Calcium influx leads to inflammation and cell death, and has been implicated in pulmonary complications and heart failure. We are supporting the development of a new therapy that has the potential to inhibit calcium influx. We have funded critical preclinical work this year, including studies in mice to help determine the optimum dose for human clinical trials.

BIGLYCAN is a natural component of muscle that activates utrophin, a protein that can step up to strengthen muscles when dystrophin isn't present. Tivorsan Pharmaceuticals is developing a recombinant biglycan discovered in Dr. Justin Fallon's lab at Brown University. Mice treated with this therapy show improved muscle function with no signs of toxicity to date. "We have made significant progress on the manufacturing/process development front," reports new CEO Jim Connolly. "Our efforts have resulted in a purified form of biglycan that increases utrophin levels 3-5 fold." Human clinical trials are slated to begin in early 2017.

SPEEDING THE SCIENCE

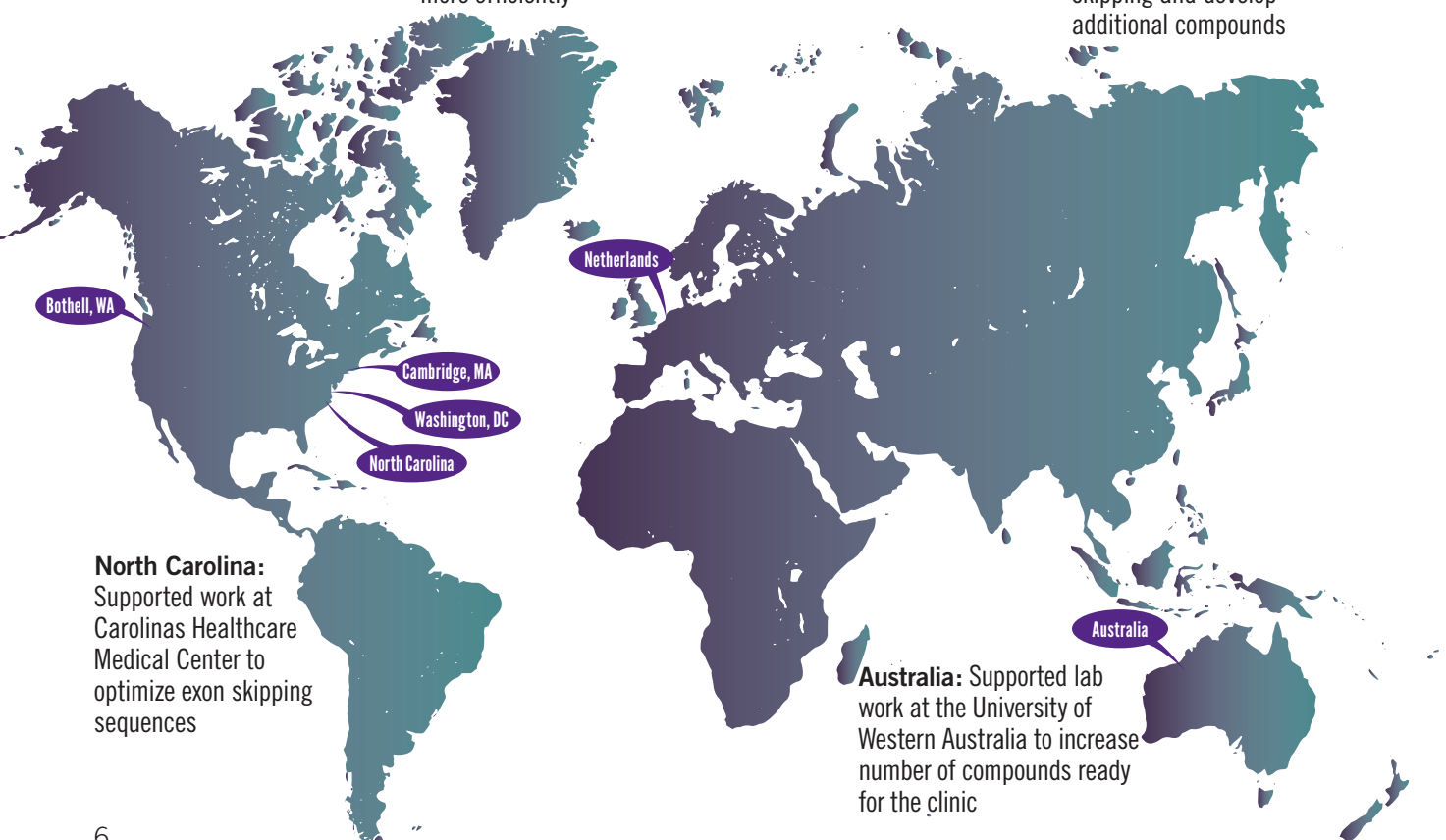
BRINGING THE FIRST EXON SKIPPING THERAPIES TO THE FDA'S DOORSTEP REQUIRED A DECADE OF DEDICATION AND A SCIENTIFIC JOURNEY THAT TRAVELED THE GLOBE

Bothell, WA: Provided support to AVIBiopharma (now Sarepta) to advance exon skipping platform

Washington DC: Pushed FDA and Congress to make Duchenne a priority and to review new drugs faster and more efficiently

Cambridge, MA: Continuing our collaboration with Sarepta to get this promising therapeutic approach to more children

The Netherlands: Financed work at Prosensa (now BioMarin) to optimize systemic delivery of exon skipping and develop additional compounds



North Carolina: Supported work at Carolinas Healthcare Medical Center to optimize exon skipping sequences

Australia: Supported lab work at the University of Western Australia to increase number of compounds ready for the clinic

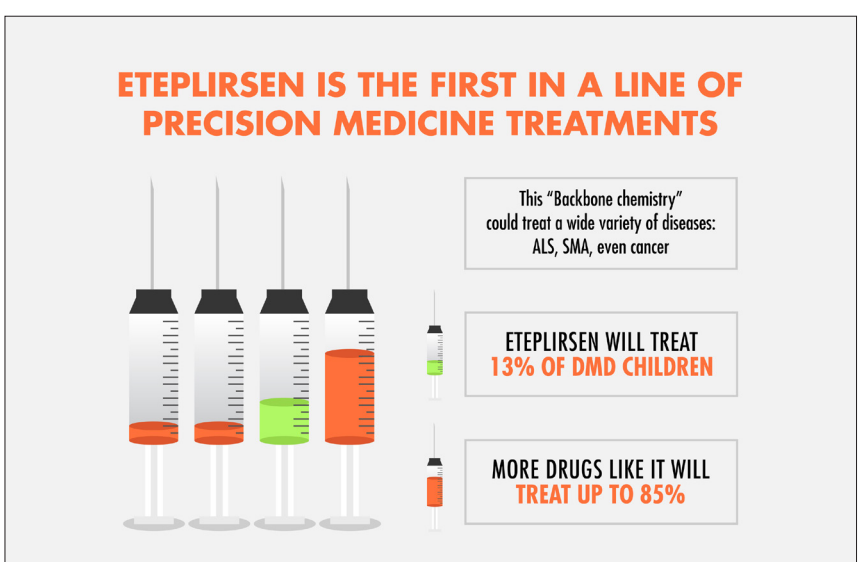
WHATEVER IT TAKES!

FROM FUNDING MULTI-MILLION DOLLAR RESEARCH PROGRAMS TO FIGHTING FOR A FASTER, BETTER DRUG APPROVAL PROCESS, WE FIND A WAY TO GET IT DONE!

We are preparing for a critical milestone in Duchenne history. Finally, after more than a decade of research and development, it is time for an FDA advisory committee to examine the first two treatments up for FDA approval. The "ad comms" will recommend whether to approve two new medicines for use by all eligible children.

It has been a long and arduous path with many difficult hurdles to clear. Along the way, Charley's Fund has stepped up to the plate to help clear the various roadblocks that have slowed progress. We have provided nearly $5 million of funding since 2005 to help advance exon skipping. We've engaged the FDA, the drug companies, and scientific experts in robust dialogue to speed the process. We launched the Race to Yes — a movement 100,000 strong — to advocate for a faster regulatory process. Our job is not done until all children with Duchenne have access to a cocktail of safe and effective therapies, but hopefully within the next few months we will be celebrating a huge first step in that direction!

These new exon skipping drugs address a specific genetic mutation, an exciting leap forward in a new era of personalized medicine. However, they only treat 13% of kids with Duchenne, a group that does not include Charley. This is a crucial first step toward a better future for all kids with DMD, but our work is still cut out for us to make sure all of us who are still waiting get access to new medicines as soon as possible.



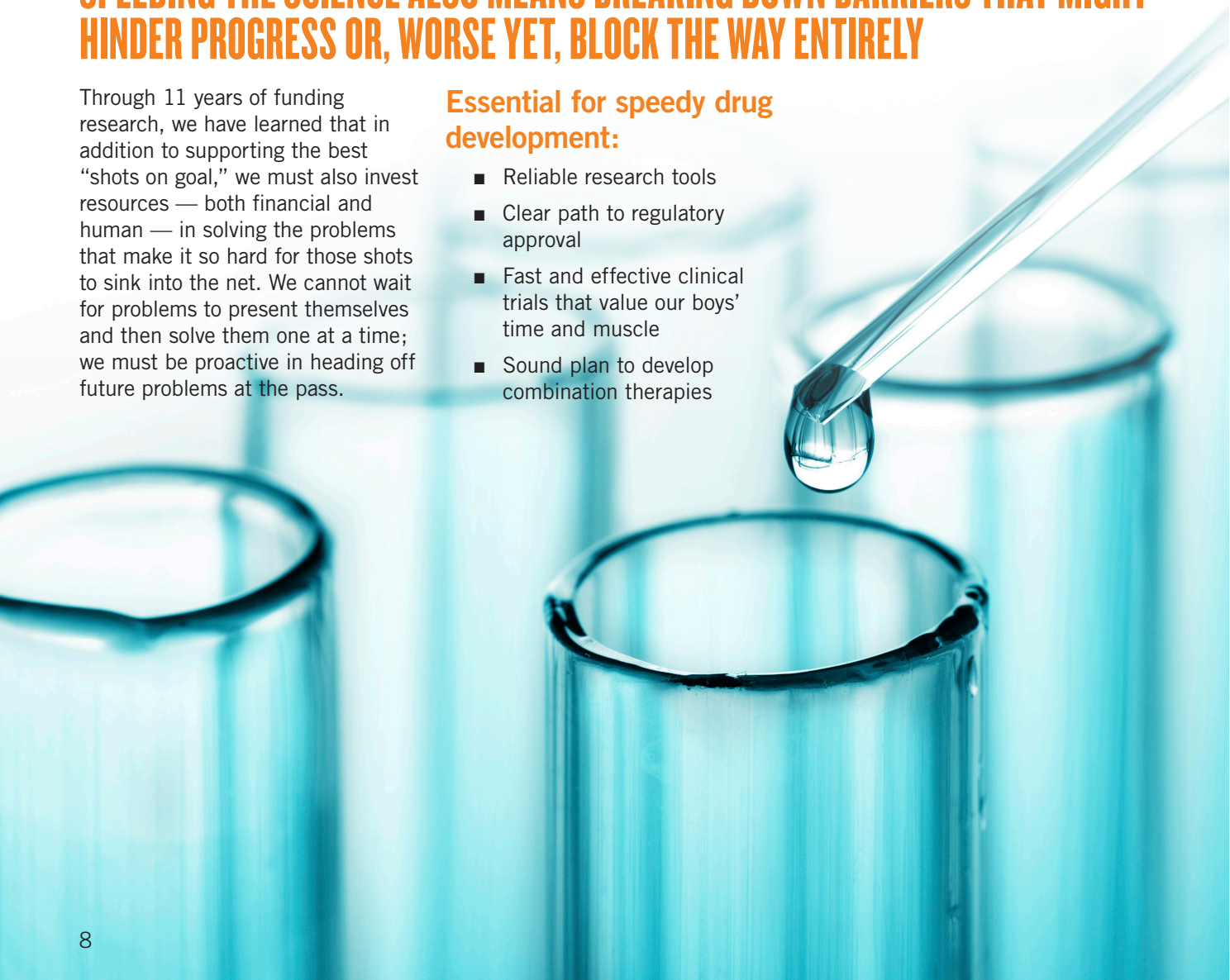
REMOVING RESEARCH ROADBLOCKS

SPEEDING THE SCIENCE ALSO MEANS BREAKING DOWN BARRIERS THAT MIGHT HINDER PROGRESS OR, WORSE YET, BLOCK THE WAY ENTIRELY

Through 11 years of funding research, we have learned that in addition to supporting the best "shots on goal," we must also invest resources — both financial and human — in solving the problems that make it so hard for those shots to sink into the net. We cannot wait for problems to present themselves and then solve them one at a time; we must be proactive in heading off future problems at the pass.

Essential for speedy drug development:

- Reliable research tools
- Clear path to regulatory approval
- Fast and effective clinical trials that value our boys' time and muscle
- Sound plan to develop combination therapies



MEET THE IMPACT INITIATIVE

A NEW PROGRAM WE LAUNCHED IN 2015 TO IDENTIFY AND REMOVE ROADBLOCKS THAT HINDER SPEEDY AND EFFICIENT DRUG DEVELOPMENT

Impact Initiative At Work: Project Goldilocks

The most oft-used animal models in DMD are not accurate enough predictors of whether a compound will have a beneficial effect in children. The "mdx mouse" has a form of muscular dystrophy that is much more mild than the human form of Duchenne, and another frequently-used model, the "double knockout mouse," has a disease form that is too severe.

Enter "Goldilocks," a new model we are testing that may be *just right*. This new model has the potential to better predict which drugs will work in humans.

Impact Initiative At Work: "Dystrophin — A Review of the State of the Science"

Several therapies in development aim to produce dystrophin, the protein boys with DMD are missing. However, once it is demonstrated that an experimental treatment produces dystrophin, it is not necessarily smooth sailing to access for all who need it. Questions can be raised by this approach, and — if not answered to the satisfaction of regulators — hold up the process.

- How much dystrophin is needed to result in clinical benefit?
- What is the best way to measure dystrophin?
- Can a child with advanced disease benefit from the production of dystrophin?

When these questions arose, Charley's Fund engaged a broad group of experts to establish an objective assessment on the topic as a reference for regulators, biotech companies and academic researchers: "Dystrophin: A Review of the State of the Science." The paper has been taken into account by FDA decision makers and praised by well-respected experts.

Jeff Chamberlain, Ph.D., University of Washington Professor of Neurology, has positive feedback on the work: "I read over the paper and thought it was fantastic! It was really well researched and written. I agree with all the conclusions and think it will be an invaluable paper for the field and to influence regulatory policy."

RAISING FUNDS AND FRIENDS

MATT'S PROMISE BENEFIT

The 10th anniversary Matt's Promise benefit was a blowout success! Highlights of the evening: Charley danced on stage with Snoop! The two hottest auction items sold not once but twice! DMD mom Mindy Leffler described how Charley's Fund helped move the FDA to action so her son Aidan could get access to eteplirsen.

(left) Charley and Snoop at the Matt's Promise benefit
(right) CEO of Matt's Promise, Randy Reiff, at the podium

CHARLEY RIDE 2015

The annual Charley Ride is the best cycling event on the Berkshire calendar. Thanks to a record number of riders, a group of committed volunteers and our generous corporate sponsors, the 2015 ride turned out to be a picture perfect day — and our biggest fundraising year yet.

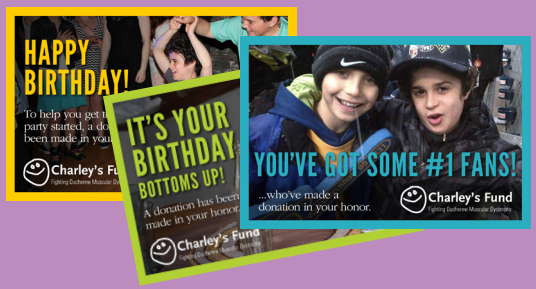
(left) Charley Ride cyclists Maria Sirois and Dan Mazursky
(right) Akashi CEO Marc Blaustein and family

THE RACE AGAINST TIME 2015

Now in its sixth year, The Race Against Time was anything but "same old, same old" in 2015! First off, our Signature Sponsor, The Shkreli Foundation, committed $150,000 to match every dollar raised and then some. Next, we shook things up with a Race the Clock Scavenger Hunt in Central Park that ended up being a total blast for all participants. Also new this year, teams around the country from New Orleans to Columbus, OH, got involved to help make this a record fundraising year.

SPECIAL OCCASIONS

Throughout the year many of our dedicated supporters honor holidays, birthdays, and milestones by making donations in honor of someone special. Now it's even easier to honor an important moment — check out our new eCards at charleysfund.org. Feeling super-ambitious? Try your hand at a fundraiser! Anything goes, from old standbys like a lemonade stand to running a 5k to just about anything you can brainstorm. We love creative ideas! If you're under 18 and raise $350, you'll earn a spot in our Hall of Fame.



WE CAN'T DO IT WITHOUT YOU

HELP US RAISE $10 MILLION TO FINANCE OUR AMBITIOUS RESEARCH AGENDA

We need to raise $10 million over three years to push the most promising therapies forward and to implement field-wide improvemens that will speed up all Duchenne research. Please dig deep this year to help us double down.

DOUBLE DOWN
to Defeat Duchenne

Your support is a key ingredient

Advancing promising therapies through the pipeline and strengthening infrastructure so these medical innovations can move quickly is expensive. But the rewards are tangible — just look at the faces of Aidan, Nash, Pietro, Nathan (see page 4) and Charley — who have access to experimental medicines because of *you*!

Help us grow the family

Charley's Fund uses popular social media sites to keep our supporters and friends up to date on the latest news.

Follow us on twitter:

@momofcharley...for updates on Charley's unique approach to life
@charleysfund...for research and fundraising news
@theracetoyes...for the latest on the drug approval process

And Like us on Facebook!

charleysfund.org

Samantha Ben-Ari (left) asked for Charley's Fund donations instead of gifts on her birthday — two years in a row!

Izzy's "Dress Down Day" is a perfect example of creative fundraising that anyone can implement to make a difference for Charley's Fund.

Charley's Fund smiley face logo makes tasty cookies more fun! Inset, Emma Goodman's benefit bake sale.

CHARLEYSFUND.ORG

him

get to know Charley and boys with Duchenne

us

learn how Charley's Fund is working with researchers, companies, and the FDA to develop therapies and a cure

you

take action, get inspired, and participate in our work for a cure

MISSION: to accelerate the development of life-saving treatments for Duchenne muscular dystrophy. We support the most compelling medical research and drive new solutions to translate promise into results.

Charley's Fund

Fighting Duchenne Muscular Dystrophy

Charley's Fund (EIN #20-2014968) is a 501(c)(3) tax-exempt public charity.