



The Heart of Our Mission: Cure: Research Breakthroughs Across Diseases......4 Care: Caring for Kids and Adults from Day One8 **Champion:** Supporting Families in Hometowns

The Heart of Our Mission: Accelerating Progress for Families

A message from



R. Rodney Howell, M.D. Chairman

MDA Board of Directors

Steven M. Derks
MDA President & CEO

At MDA, families are at the heart of all we do. We are guided every day by our mission to free individuals — and the families who love them — from the daily challenges and life-threatening effects of muscular dystrophy, ALS and related diseases.

Each day across the country, everyday freedoms like walking, hugging, brushing one's teeth, getting dressed and even breathing are taken away from kids and adults by neuromuscular diseases. MDA is fighting to give strength, independence and life to every child and adult affected so they can live longer and grow stronger.

Significant and exciting progress is underway, as is evidenced by the encouraging advances you'll see documented in this report on MDA's operations and activities during 2015:

- Research Progress Across Diseases: This section highlights 2015 research advances that were made possible in part by MDA's long-term investment in neuromuscular disease research.
- Caring for Kids and Adults from Day One: Here you will see MDA's
 efforts during 2015 to care for families from the moment of diagnosis
 throughout their entire journey by optimizing health, quality of life and
 independence.
- Supporting Families in Hometowns Across America: This section highlights the impact MDA's life-enhancing services and support programs had on families in communities across the country in 2015.

Muscle-debilitating diseases continue to impose enormous physical, emotional and financial challenges on individuals and their families. We're committed to thinking bigger, working harder and accelerating the rapid progress currently underway.

We are extraordinarily grateful to every individual, company, team, organization and family who is making our shared progress possible. Together, we are moving forward in partnership with families, researchers and clinical partners to translate hope and progress into urgently needed answers and tangible results MDA families are counting on.

Cure: Research Breakthroughs Across Diseases

"A really important thing MDA does is not only push current therapies, but they spend a lot of money in trying to bring along the next generation of scientists. That's critically important because research funding is drying up across the world, and a lot of young people today are discouraged from going into scientific and medical research."

Jeffrey Chamberlain, Ph.D.,
 MDA Research Advisory
 Committee member



Research Breakthroughs Across Diseases

At MDA, we take a big-picture perspective across the full spectrum of neuromuscular diseases to uncover breakthroughs that accelerate treatments and cures. The power in our research approach is that we often can apply learnings from one disease to achieve progress in others — and bring urgently needed answers to families.

Since MDA's inception more than 60 years ago, we've funded groundbreaking research and fostered communication and collaboration among scientists across the full spectrum of diseases.

This innovation and collaboration is leading to the development and imminent availability of new lifesaving therapies. By partnering with the world's top researchers, biotech and pharmaceutical organizations, and families who play an essential role in clinical trials, MDA's research efforts in 2015 brought us a significant step closer to new answers and discoveries.



Research Breakthroughs Across Diseases



\$16.4

million

Total 2015 research dollars invested



More than

150

Research projects funded in 11 countries



390

MDA-funded research projects active during 2015, with total funding commitment of \$92.9 million



46

ALS research grants active during 2015, with total funding commitment of \$12.1 million



103

New grants awarded in 2015, with a total funding commitment of \$27.3 million



Nearly

200

Clinical trials underway for diseases in our program, testing dozens of promising therapies.



Research Breakthroughs Across Diseases

"Building a foundation of medical research requires early exciting studies, many of which are supported by MDA."

Jeff Rothstein, M.D., Ph.D.,
 MDA Clinical Advisory
 Committee member

Research Progress in 2015

Noteworthy research advances supported by MDA funding:

- The exon skipping drug eteplirsen (brand name Exondys 51), under development to treat some forms of DMD, is under review by the FDA. [Note: Eteplirsen (Exondys 51) was granted accelerated approval by the FDA in September 2016.]
- Keveyis becomes the first FDA-approved drug for the treatment of hyperkalemic and hypokalemic periodic paralysis.
- MDA, Genzyme and Emory University team up to expand access to genetic testing for accurate diagnosis of limb-girdle muscular dystrophy.
- Transport between the nucleus and cytoplasm was shown to be disrupted in ALS, yielding novel drug targets and improving our understanding of the disease.
- Ionis Pharmaceuticals launches a phase 1 trial to test antisense therapy for ALS.
- Dystrophin gene editing, using a strategy known as CRISPR-Cas9, continues to show promise to treat DMD in cultured cells and mice.
- American Academy of Neurology and American Association of Neuromuscular and Electrodiagnostic Medicine release a guideline for the diagnosis and care of people with facioscapulohumeral muscular dystrophy.
- Reveragen BioPharma launches phase 1, first-in-human clinical trial to test DMD drug vamorolone in healthy volunteers.
- Updated American Academy of Neurology care guidelines for physicians caring for children with congenital muscular dystrophies.
- In clinical trials sponsored by Biogen and Ionis Pharmaceuticals, infants with SMA who were treated with nusinersen show improved measures of muscle function.



Care: Caring for Kids and Adults from Day One

"Getting our daughter's diagnosis when she was 2 years old was scary, shocking and isolating. Right off the bat, we knew MDA was on our team and would be there to support us. To have an organization there to provide a community and be a resource for us was and continues to be a huge asset for our entire family."

 Becky Bormann, whose daughter has congenital muscular dystrophy



Caring for Kids and Adults from Day One

Advancing Care for Families

Early diagnosis, highly specialized care and access to promising clinical trials help ensure the best possible outcomes for individuals and families.

MDA cares for kids and adults from day one at our network of MDA Care Centers across the United States and Puerto Rico. MDA Care Centers offer families best-in-class, comprehensive care. These state-of-the-art clinics, located at the top hospitals and medical facilities, bring health care specialists from a variety of disciplines together so families receive the care they need at one time and in one place.

These experienced clinicians have a depth of knowledge that allows them to recognize subtle differences between conditions that may resemble each other at onset, but which have very different underlying causes, rates and patterns of disease progression, and standards of care.

MDA Care Centers receive nearly 50,000 visits each year while also serving at the forefront of research by hosting clinical trials for the latest promising therapies.



Caring for Kids and Adults from Day One







More than

150

MDA Care Centers across the United States and Puerto Rico

43

MDA ALS Care Centers



400

Neuromuscular disease researchers and experts who shared knowledge at MDA's 2015 Scientific Conference



"MDA means hope. It means strength. It means courage. MDA has been great helping [our child] overcome everyday limitations, and I can't be more thankful."

> Josh Lybrand, whose son has Duchenne muscular dystrophy



Supporting Families in Hometowns Across America

Progress in Supporting and Empowering Families in 2015

MDA is here for families in hometowns across America, ready to assist and empower the kids and adults we serve to help them thrive and maintain independence.

From offering support groups and educational seminars that help caregivers, parents and individuals through their journey — to connecting families with information when and where they need it and giving kids with muscular dystrophy and related diseases the best week of the year at MDA Summer Camp, MDA is here to help families maintain and improve their health and well-being while actively pursuing life goals to live unlimited.



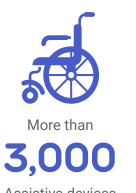
Supporting Families in Hometowns Across America











Assistive devices provided to MDA families



9,500

Visitors to **mda.org** every day



More than

100

support groups and educational gatherings in 2015



Supporting Families in Hometowns Across America

Making Families' Voices Heard in Public Policy & Advocacy

MDA is dedicated to doing everything in our power to advocate for policies and programs that help save and improve the lives of kids and adults living with muscular dystrophy and related neuromuscular diseases. Together, we ensure that our community's collective voice is heard.

Every year, MDA works closely with Congress, regulatory agencies and other leaders to ensure that issues impacting the neuromuscular disease community are a priority. MDA and our advocates fight for life-changing public policies, and we are proud to collaborate with other organizations and stakeholders in the disability community to ensure the passage and implementation of policies and programs supporting the families MDA serves.



Supporting Families in Hometowns Across America

In 2015, MDA and its community of advocates — including MDA families, leaders, scientists and clinical experts — supported the following legislation and had a pivotal role in the community-wide effort that led to these advances.

Ensuring Access to Clinical Trials Act of 2015



Signed into law Oct. 7, 2015, this legislation makes it possible for individuals to participate in rare disease clinical trials and receive up to \$2,000 in compensation without the funds counting against income eligibility for Medicaid and Social Security Income (SSI). The collaborative effort, led by the Cystic Fibrosis Foundation in partnership with the National Organization for Rare Disorders and MDA, was supported by more than 75 organizations who worked to ensure the legislation passed.

Increase Funding for the National Institutes of Health (NIH)



MDA worked to increase funding for the National Institutes of Health (NIH), the largest funding source of biomedical research in the United States. In December 2015, Congress approved the largest funding increase — an additional \$2 billion — in more than a decade. This funding increase translates into more federal funding for research that will help accelerate treatments and cures for the conditions in MDA's program.

Preserve Access to Complex Rehabilitation Technologies



As part of an organized coalition, MDA supported legislation preventing cuts to the Medicare program that would limit access to life-enhancing complex rehabilitation technology (wheelchair accessories) that individuals with disabilities rely on every day to maintain independence, mobility and optimal health. Congress passed a bill in December 2015 delaying the cuts for 12 months. MDA continues to advance this critical issue.

Led Collaborative
Effort to Inform
FDA About
Biomarkers to
Promote Therapy
Development



MDA led a collaborative effort to provide the U.S. Food and Drug Administration (FDA) with biomarker information about disorders in MDA's program. MDA provided current scientific data about biomarkers and how those measures could be used in the drug and therapy development process for multiple neuromuscular diseases. These markers, or signals, can provide crucial information for researchers conducting clinical trials, helping them determine early on whether a treatment is effective.



Partners in **Progress**

In 2015, MDA continued to find ways to innovate our fundraising programs to fuel our lifesaving mission. The highest standards of careful stewardship and fiscal transparency were applied across the management of MDA's financial operations to ensure stability and vitality for our lifesaving work.

Together with our generous partners and supporters, we continue to work to help families find much-needed hope, answers and support, whenever and wherever they need us. Since MDA's founding in 1950, our strength and our hope lie in the hearts of the people and partners who embrace our mission.



Partners in Progress

Partnerships Powering **Progress**

For decades, MDA has been proud to team up with caring corporations, organizations and brands that generate tens of millions of dollars each year through creative campaigns, sales promotions and year-round special events.

We are grateful to the following partners whose efforts and generosity each helped generate more than \$1 million during the past year to help families with muscular dystrophy and related life-threatening diseases.



















The International Association of Fire Fighters

(IAFF) committed by proclamation in 1954 to support MDA until a cure is found. During its 60-year partnership with MDA, the IAFF has raised a staggering \$583.5 million for MDA families. The organization's commitment has remained rock-solid, as more than 100,000 dedicated fire fighters across our nation dedicate time every year to raise money on street corners and visit kids at MDA Summer Camp. In 2015, the IAFF contributed more than \$25 million through more than 2,000 Fill the Boot events and other special fire fighter events to benefit MDA.

As MDA's largest corporate partner, CITGO Petroleum Corporation and its nearly 5,500 locally owned retail locations have raised more than \$180 million for MDA through a wide variety of fundraising events, including golf tournaments, fundraising at checkout and socials. In 2015, CITGO contributed nearly \$15 million to support MDA's lifesaving mission.

Lowe's Home Improvement, which has contributed more than \$57 million since partnering with MDA in 2001, teamed up with customers in 2015 at more than 1.700 Lowe's locations to achieve more than \$7.5 million through sales of MDA Shamrocks. Dedicated Lowe's employees also volunteer at MDA Summer Camps across the country, helping kids develop lifelong friendships, build self-confidence and enjoy a week of barrier-free fun.

Harley-Davidson Motor Company, which has put its commitment to freedom and independence into action by supporting MDA since 1980, raised more than \$3 million for MDA in 2015 through events such as Black-N-Blue Galas and special MDA rides, including the EHDDA MDA Ride for Life in Pennsylvania. During its 35-year partnership with MDA, the Harley-Davidson family of dealers, customers, employees, suppliers and H.O.G. chapters has raised more than \$96 million to help save and improve the lives of MDA families.





Strength in **Numbers**

MDA is proud to be a grassroots organization with a national reach. In decades past, people helped MDA in many unique and innovative ways, from holding backyard carnivals to riding in bike-a-thons and collecting pennies to bring to local telethons.

In 2015, that incredible spirit of generosity and involvement was as vibrant as ever and visible in a variety of special events that generated funds and raised awareness for MDA's lifechanging mission.

MDA's cause is strengthened by our army of more than 350,000 volunteers in hometowns across America. Their tireless efforts and passionate support translate into an unparalleled commitment to helping MDA accelerate progress in research, care and support for our families.



More than

30,000

retailers selling Shamrocks and participating in other customer-ask promotions



350,000

volunteers



participants



local events through our 100 offices (total MDA fundraising events in 2015)





MDA Events: Strength in Numbers



MDA Muscle Walk

MDA Muscle Walk is a life-changing event that inspires and strengthens families and communities. Muscle Walk is more than a fundraising walk. For MDA families, friends, volunteers, donors and sponsors, it's a powerful experience that forges lifelong connections, celebrates families and the barriers they overcome and turns hope into answers. In 2015, about 40,000 people participated in 150 MDA Muscle Walks, raising \$8 million to support the families we serve. Since 2011, MDA Muscle Walk has raised more than \$34 million to help bring strength to life for MDA families in hometowns across America.



Fill the Boot

For more than 60 years, our nation's heroes have been collecting donations — one dollar at a time in their boots — from generous motorists, shoppers and neighbors through Fill the Boot events. More than 100,000 fire fighters across the country participate in this spirited tradition each year. In its first year in 1953, fire fighters in Boston raised \$5,000 for MDA. In 2015, more than \$25 million was raised by fire fighters at more than 2,000 Fill the Boot events and other local events. To aid in their efforts, MDA launched Fill the Boot Online in August 2015 to assist fire fighters in collecting online donations, which raised more than \$11,000 in four short months.



MDA Shamrocks

From early February through the end of March, MDA's iconic St. Patrick's Day fundraiser is supported by more than 25,000 retail locations across the country — including supermarkets, convenience stores, restaurants and other community-minded businesses. Retailers sold paper Shamrocks (scannable cards) at checkout, and customers purchased the paper Shamrocks for \$1, \$5 and larger contributions for the retailer to display in support of MDA. Among our dedicated supporters were Lowe's Home Improvement, Burger King, CITGO Petroleum Corp., Kroger and 7-Eleven. Shamrocks raised nearly \$18 million in 2015. The paper Shamrocks continue to stand as a symbol of strength, independence and life.

For Strength, Independence & Life 21 Back to Table of Contents

MDA Events: Strength in Numbers



MDA Lock-Up

MDA Lock-Up is a fun and inspiring community event that unites business leaders to raise funds and awareness to help kids and adults break free from the harm of muscle-debilitating diseases. Business leaders joined forces with MDA in neighborhoods across America, taking part in MDA Lock-Up events. Jailbirds agreed to be "locked up" while they raised money for their "bail." In 2015, MDA Lock-Up events raised nearly \$14 million nationwide.



MDA Distinguished Events

Galas, golf tournaments and other signature events -362 total - in 2015 raised \$17.8 million to help MDA families.



MDA Team Momentum

MDA Team Momentum offers beginners and endurance pros alike the opportunity to participate in a marathon or half marathon while raising funds to accelerate progress for MDA families. In its second year, MDA Team Momentum — and its more than 600 participants — raised more than \$850,000 to help MDA families. In its first two years, MDA Team Momentum had a total of 954 participants and raised a combined \$1.55 million.



Your Way for MDA

Launched in June 2015, Your Way for MDA is an online platform that gives MDA supporters the opportunity to decide how they'll raise money for kids and adults in their community and hometowns across America. Your Way gives the American public the choice and tools to raise money in the most creative ways — from hosting a bake sale to running a 5K to an activity that speaks to your unique story. Thanks to the public's creative ideas, Your Way for MDA raised \$117,707 through the end of 2015.



Volunteer Leaders

2015-2016 Officers

(one-year term beginning July 15, 2015)

Chairman, Board of Directors

R. Rodney Howell, M.D. Miami, Fla.

Vice Chair, Board of Directors

Christopher J. Rosa, Ph.D. New York, N.Y

Secretary

Charles D. Schoor, Esq. Valley Village, Calif.

Treasurer

Victor Wright New York, N.Y.

2015-2016 Directors

(one-year term beginning July 15, 2015)

Stanley Appel, M.D. Houston, Texas

C. Thomas Caskey, M.D.

Houston, Texas

Harold Crump St. Paul, Minn.

Benjamin Cumbo III Upper Marlboro, Md.

Steve Farella New York, N.Y.

Daniel Fries New York, N.Y.

Honorable Brad Henry Norman, Okla.

Dave Hutton Valencia, Calif.

Louis Kunkel, Ph.D. Boston, Mass.

Patricia Nazemetz Sleepy Hollow, N.Y.

Mike Rowlett

Farmers Branch, Texas

Mark Smith Houston, Texas

John Tognino Ardsley, N.Y.

Kristine Welker Hartsdale, N.Y.

Lilian Wu, Ph.D. Armonk, N.Y.



Volunteer Leaders 2015-2016 National Vice Presidents (one-year term beginning July 15, 2015)

Volanicoon	Loadol o 20	10 2010 Nacional V	Tee i residentes (one	year term degirining ou
Arizona	District of Columbia	Massachusetts	Scott Masterson	Dave Carroll
Todd Bresnahan	Fredric Rolando	Robert Sigel	John McGinley	Peter Dectis
Derrick Hall	Harold Schaitberger	Minnesota	Hiroshi Mitsumoto, M.D.	Mike Dunleavy
California	Florida	Marc Moeller	Natalie Morales	Kara Fox-LaRose
Brandon Barash	Jay Feely	Missouri	Ted Moudis, AIA	Joe Gentile Jr.
Todd Beck	Mario Kreutzberger	Don Breckenridge Jr.	Mike Neary	Dan Hilferty
Alexander Cappello	Georgia	Gary Drewing	Lewis Rowland, M.D.	Jack Krol
Jann Carl	Bruce Lucia	Nevada	Ray Tierney	Puerto Rico
Frank DiBella	Tom Robinson	Jim Prather	Lisa Utasi	Alan Cohen
Evan Lamberg	Jack Markwalter Jr.	New Jersey	North Carolina	Juan Larrea
S. Paul Musco	Illinois	Kevin Boothe	John Clark	Aniceto Solares
Nancy O'Dell	Chris Clawson	Anthony Cammarata Jr.	Clifton Rutledge	South Carolina
Larree Renda	William Emmons	John Crowley	Kevin Urban	Randy Kibler
Bert Selva	Chris Ondrula	Chris Snee	Ohio	Tennessee
Alison Sweeney	lowa	New Mexico	Maureen McGovern	Pete Fisher
Tom Thomas	Reynolds Cramer	William Anderson	John Quinlan, M.D.	Bill Mayne
Ace Young	Robert Myers	New York	Oklahoma	Texas
Colorado	Kansas	Candace Beinecke	Susannah Adelson	Eric Affeldt
Jake Jabs	Richard Seithel	Vincent DeLazzero	Jim Brown	Bill Breetz
Connecticut	Maryland	Charles Fazzino	Nadia Comaneci	Stuart Crum
Richard Graziano	Lon Rosenberg	Steve Furnary	Oregon	Rusty Hardin
Lynn Malerba	John Seabers	Lel and Tom Gimbel	Mike Bellotti	Bill Klesse
	Barry Sheaffer	Neil Golub	Pennsylvania	Pierce Marshall
	Jennifer Smith Stepanek	James Halpin	Ty Ballou	Mike Withers
		Michio Hirano, M.D.	DeLight Breidegam	Wisconsin

Michio Hirano, M.D.

Gordie Boucher Sr. Back to Table of Contents

Volunteer Leaders

Clinical Advisory Committee

(members as of June 26, 2015)

Yaacov Anziska, M.D.

Susan Apkon, M.D.

Jan Bonner

Joline Dalton, CGC

Richard Finkel, M.D.

Daragh Heitzman, M.D.

Neil Holland, M.D.

Tomas Holmlund, M.D.

Irwin Jacobs, M.D.

Wendy King, PT

John Kissel, M.D.

Katherine Mathews, M.D.

Dennis Matthews, M.D.

Tahseen Mozaffar, M.D.

Jeffrey Rothstein, M.D., Ph.D.

Barry Russman, M.D.

Ericka Simpson, M.D.

Jonathan Strober, M.D.

Research Advisory Committee

(members as of January 1, 2015)

Chairmen

Stanley Appel, M.D.

Louis Kunkel, Ph.D.

Members

Robert Baloh, M.D., Ph.D.

Elisabeth Barton, Ph.D.

Kurt Beam, Ph.D.

Alan Beggs, Ph.D.

Sanjay Bidichandani, MBBS, Ph.D.

Mark Bromberg, M.D., Ph.D.

Dean Burkin, Ph.D.

Jeffrey Chamberlain, Ph.D.

M. Casey Childers, D.O., Ph.D.

Thomas Crawford, M.D.

Merit Cudkowicz, M.D., M.Sc.

John Day, M.D., Ph.D.

James Dowling, M.D., Ph.D.

Dongsheng Duan, Ph.D.

Heather Durham, Ph.D.

Richard Finkel, M.D.

Emanuela Gussoni. Ph.D.

Kenneth Hensley, Ph.D.

Michio Hirano, M.D.

Bernard Jasmin, Ph.D.

Joe Kornegay, D.V.M., Ph.D.

Rashmi Kothary, Ph.D.

Jun Li, M.D., Ph.D.

Catherine Lomen-Hoerth, M.D., Ph.D.

Christian Lorson, Ph.D.

Giovanni Manfredi, M.D., Ph.D.

Nicholas Maragakis, M.D.

Katherine Mathews, M.D.

Elizabeth McNally, M.D., Ph.D.

Lynn Megeney, Ph.D.

Matthew Meriggioli, M.D.

Daniel Miller, M.D., Ph.D.

Carlos Moraes, Ph.D.

Kanneboyina Nagaraju, D.V.M., Ph.D.

Sally Nelson, Ph.D.

M. Kerry O'Banion, M.D., Ph.D.

Bradley Olwin, Ph.D.

Robin Parks, Ph.D.

John Ravits, M.D.

Michael Shy, M.D.

Shanthini Sockanathan, Ph.D.

Melissa Spencer, Ph.D.

Maurice Swanson, Ph.D.

C. Chris Weihl, M.D., Ph.D.

Ex officio member

R. Rodney Howell, M.D.



Volunteer Leaders

Registry Advisory Board

(members as of June 26, 2015)

Alan Beggs, Ph.D.

Joshua Benditt, M.D.

James Berry, M.D.

Thomas Crawford, M.D.

Kevin Flanigan, M.D.

Eric Hoffman, Ph.D.

Emily Munson, Esq.

Rachel Richesson, Ph.D., MPH

Jeffrey Rosenfeld, M.D., Ph.D.

Carly Siskind, M.S., CGC

Consultants

Julie Bolen, Ph.D., MPH

Amelie Gubitz, Ph.D.

John Porter, Ph.D.

MDA Venture Philanthropy Advisory Committee

(members as of January 1, 2015)

Stanley H. Appel, M.D.

Cristina Csimma, PharmD, MHP

Kenneth H. Fischbeck, M.D.

Amelie Gubitz, Ph.D.

John E. Howell

Louis M. Kunkel, Ph.D.

Elizabeth McNally, M.D., Ph.D.

Thomas A. Rando, M.D., Ph.D.

Jeffrey D. Rothstein,

M.D., Ph.D.

Lee Wrubel, M.D.



Financial Report For the year ended December 31, 2015

(in thousands)

Assets

\$87,787
5,590
869
94,246

Liabilities

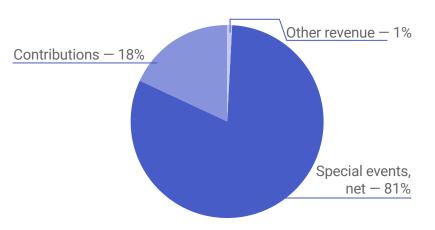
Accounts payable and accrued expenses	5,303
Research awards and grants payable	11,487
Line of credit	14,500
Pension and post-retirement plan obligations	54,536
Total liabilities	85,826

Net Assets

Net Assets	
Unrestricted	3,908
Temporarily restricted	4,028
Permanently restricted	484
Total net assets	8,420
Total liabilities and net assets	\$94,246

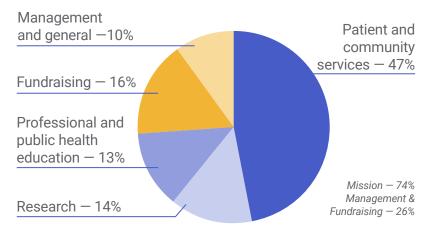
Revenue

Special events, net	\$99,375
Contributions	22,336
Other revenue	474
Total revenue	\$122,185

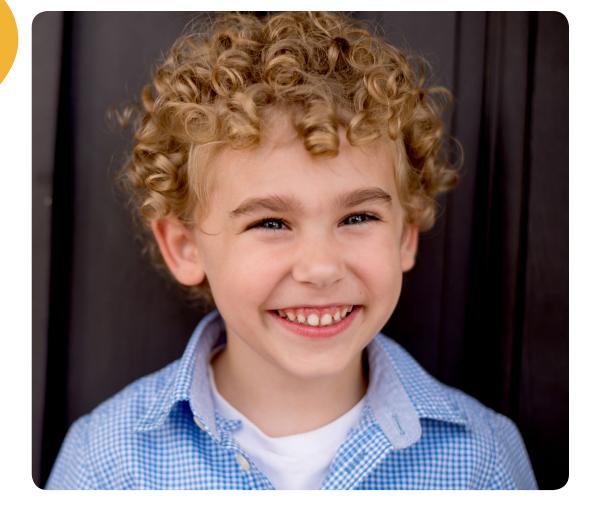


Expenses

Patient and community services	\$56,468
Research	17,150
Professional and public health education	14,963
Fundraising	19,741
Management and general	12,824
Total expenses	\$120,811







For Strength, Independence & Life

Muscular Dystrophy Association







f facebook.com/MDAnational



