



**NEUROMUSCULAR
DISEASE FOUNDATION**

JOIN THE GLOBAL EFFORT TO CURE NEUROMUSCULAR DISEASES

2019 Annual Report & Impact Statement

Review of the 2018 Calendar Year

A Message from the CEO

Dear Supporters,

2018 was a year of unprecedented growth and opportunity for NDF, its patients, and scientists. We received a transformative matching grant from a philanthropic organization committed to cutting edge science, and it was humbling to see our community rally to make accepting it legally viable.¹ It would be fun to say that our most successful gala in NDF's history raised a million dollars in one night, but that wouldn't be quite true. It has taken us 4 years to foster the global and local support from you, our donors, that culminated in a beautiful night of art, music, and true philanthropy.

Our robust online presence has allowed for NDF's global reach. Not only did that reach increase our donor base by over 300%, but it has helped us grow our identified patient population by 27%. This increases our value to the biopharmaceutical industry, on whom we rely so heavily for curative therapies. I am delighted to report that the \$2M in funding we raised in 2018 has been furthering our goals through NDF symposia and other important events. These events lead to new, shared collaborations and potential treatments. Funds gifted by NDF to the NIH for ManNAc trials and our subsequent efforts in gene therapy with the FDA are a few of our promising examples.

On behalf of our Board of Directors, scientific team, staff, carriers, and patients alike, I wish to thank every one of our donors for your donations and for honoring pledges made at our gala last November. Without you, none of this success would be possible. I look forward to seeing you at our 2019 Gala on November 17th!

Sincerely,



Lalé J. Welsh, CEO/Executive Director, NDF

2018 in Review

Awareness Impact:

359%

increase in donor base

27%

growth in NDF patient registry

80%

increase in global reach – 29 countries and territories now represented

37%

increase in online followers

11

conferences attended, allowing us to forge global and strategic relationships

6

speaking engagements, establishing NDF as the leading organization dedicated to GNEM

3

biotech companies engaged as prospects for gene therapy funding

Events Impact:

1

Gala – Our most successful fundraising event to date

2

Ambassador outreach events

3

Symposia (UK, Turkey, and US) and 2 Online Seminars

45%

increase in engagement of never before reached people in regional, young adult community that is disproportionately affected by GNEM

NDF Mission

Our mission is to enhance the quality of the lives of people living with GNE Myopathy (also known as HIBM)² through advocacy, education, outreach, and funding for critical research focused on treatments and a cure.

About GNE Myopathy

GNE Myopathy—or HIBM—is a distal myopathy: a rare genetic disease starting at the feet, causing muscles to slowly weaken. GNE Myopathy is not life-threatening, but it may lead to physical debilitation within two decades of diagnosis. Symptoms usually begin to develop in early adulthood, between late teens to early 30's. GNE Myopathy exists in all races and nationalities, worldwide; however, ongoing natural history studies³ show elevated carrier rates in certain populations of Eastern European and Asian heritage; including but not limited to Jewish, Persian, Uzbeki, Arab, East Indian, Indonesian and Japanese.

Contact Us

CEO/Executive Director
Lalé Welsh

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Beverly Hills, CA 90212
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10 Bond Street
Suite 330
Great Neck, NY 11021
(516) 441-7126

Tax ID
06-1789643
Registered 501(c)(3)

Learn More

info@CureHIBM.org
CureHIBM.org

Financial Impact:

\$2,000,000
raised*

693
individual donors

3
awardees celebrated

*Including November 2018 pledges

Philanthropy Award
David Dahl



David Dahl accepting award from Jenifer Yashari

Community Impact Award
Shari Rezaei



Shari Rezaei accepting award from presenters Sussan Deyhim & Chloe Pourmorady

Advocacy Award
NDF Ambassadors



Desiree Pakravan and Sogol Ashourpour, Gala Co-Chairs representing the NDF Ambassadors

NDF has documented GNE Myopathy patients in 29 countries and territories.



- | | | | | | |
|---------------|---------|---------|-------------------------|--------------|----------------------|
| Bangladesh | England | Ireland | The Netherlands | Russia | Taiwan |
| Bulgaria | France | Israel | Northern Ireland | Saudi Arabia | Turkey |
| Canada | Germany | Italy | Pakistan | Scotland | United Arab Emirates |
| Côte d'Ivoire | India | Japan | Palestinian Territories | South Korea | United States |
| Egypt | Iran | Nepal | Portugal | Suriname | |



NDF Staff & 2018 Board Members, left to right: Ralph Loren, Nancy Lurie (NDF Director of Operations), Carolyn Yashari Becher, Carol Gelbard, Lalé Welsh (NDF CEO), Tricia Mullins, Robert Becher, Jason Taban and Michael Banayan.



2018 Gala Art Exhibition: PastForward: Through the Lens of Iranian Women

2018 Program Accomplishments

Clinical Research:

Completed funding of 2 year Haddassah Grant

Completed funding of UCLA grant for research in the Middle East

Completed funding of NIH ManNAc trial assistance grant

Begun sample collection of 2 year WGS study with PerkinElmer

Begun funding of RNA Sequencing study with Yale/Lek Lab

Begun WGS study with NIH

Identified new researchers, patients, and physicians in multiple new countries

Patient Advocacy:

Los Angeles 2 day Symposium

Addition of CPAs in Germany, Côte d'Ivoire, and Israel

Sponsored or participated in patient days in UK, Turkey, and India (WWGM)

Inaugural Online Patient Seminars

Empowered patients to self-advocate through quarterly Certified Patient Advocate meetings

Rare Disease Day Rare Reality Campaign

Featured speaker at Global Genes Patient Summit + European Neuromuscular Center Conference (ENMC)

Taught other rare disease groups how to collaborate and build their organizations

Outreach & Education

Celine Fundraising & Outreach Event in New York

Ambassadors' educational friend raising dinner

Press coverage by rare disease organizations including Global Genes, Think Genetic, Patient Worthy, Zeal Access, and The Mighty

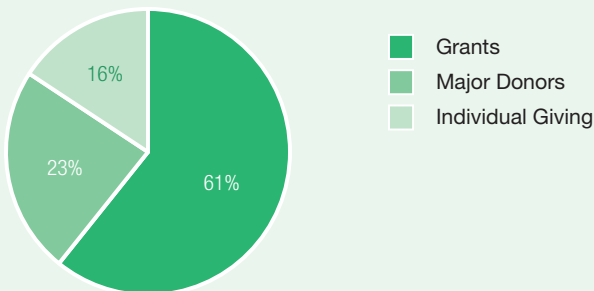
Media placement in international cultural publications

Secured coverage in radio, digital, and broadcast news outlets

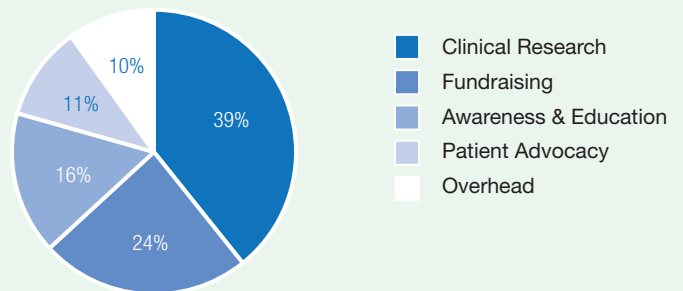
Your Donations At Work

90 cents⁴ of every dollar goes towards funding scientific research and core programs providing resources to families living with GNE Myopathy.

Income Sources



Where Your Money Goes



At our 2018 Symposium at UCLA, GNE Myopathy and NDF Patient Advocacy Program Manager, Tara Voogel accepting this Certificate of Congratulations from the Office of Los Angeles Mayor Eric Garcetti



Patients and family members from around the world gather together at NDF's 5th Annual Symposium on GNE Myopathy

Thank you to our Generous Donors

\$1,000,000 and up

Anonymous 501(c)(3) Private Foundation

Visionaries - \$100,000 and up

Michelle & Bob Etebar*

Nasser (David) Haverim*

Anna & Bijan Rodd

Benefactors - \$50,000 to \$99,999

Confidence Foundation

Marjaneh & Afshin Etebar

Ultragenyx Pharmaceutical

Patrons - \$25,000 to \$49,999

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Sherli & Mishel Mikail and Farah & Halston Mikail

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Carolyn Yashari Becher & Rob Becher

Rosita Neman & Tooraj Monsef**

Sabrina & Kamram Younesi*

Rita Neman & Shaoul Javaheri*

Sharareh & Kamran Neman**

Heroes - \$15,000 to \$24,999

Quinn Emanuel Foundation

Natalie & Michael Zarabi

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Angela & Isaac Larian

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Benjamin Kermani**

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Shirin & Peyman Yadegar

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Debbie & John Neman

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Rachelle Rojany & Jeffrey Noles

Angela & (Bob) Behzad Barouti

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Ladan & Daniel Kadisha

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David & Elishia Bolour

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Jason Keller

Ezabel & Benjamin Broukhim

Elizabeth Grace

Jason Lieberman

Sonia Cazares

Natasha & Sharon Hakakian

Jason Liebman

Roya & Peter Cohen

David Haloossim

Ralph Loren

Joan & Edward Cohen

Monica & Mark Haloossim

Houman Mahboubi

Shaheen Manshoory

Maxine & Alex Martin

Haleh & Bahman Mashian

Greg Merage

Farrah & Tahseen Mozaffar

Tricia Mullins

Elana Naghi

John & Sibelle Nazarian

Angella & David Nazarian

Rita Neman

Ramin Neman

Ronnie & Farnoush Novian

Tammy Plotkin-Oren & Arnon Oren

Azita & Nader Pakravan

NDF encapsulates hope; hope for me and the many other GNEM patients who are suffering. NDF means that I and others like me are not isolated. The staff at NDF treats all with utmost care and dignity. Most of all, they listen to the GNEM community. NDF is well positioned and well qualified to continue exploring all avenues of science to bring a cure for GNE Myopathy.

- Tara Voegel

*GNEM patient and
NDF Patient Advocacy
Program Manager*

Shohreh & Medhi Soroudi*
Jila & Benjamin Yadegar*
Kambiz Yadegar
Ramesh & Emanuel Yashari
Anonymous
Anonymous

Janet & Siamak Okhovat
Lidi Pourrabbani
Daniel Skura
Azita & Albert Taban
Lalé Welsh*
Mike Yim
Anonymous

Keyuri Patel
George & Katrin Peykar
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Tiffany & Elroy Vojdani
Niloufar & Dariush Yashari
Mahnaz & Manoochehr Yashari
Anonymous
Anonymous

Just the other day we were talking about how we use the word patients all the time. Maybe there is another, better word, for us. I did some research online and I realized that, no, there isn't a better word. Patients as a noun is someone with a medical condition, but patient as an adjective is capable, willing to endure, thoughtful, attentive, unflappable.

So, we are patients. We are capable. We are willing to endure. I am thankful for all the patients that I have connected to. I feel like you are my family. Every email I get, I will answer. Any questions you have, I will research, and try to answer for you. I am thankful for meeting all of you, all the board members, all the parents, all the caregivers, everyone. We are thankful for you.

- Amy Curran

GNEM patient and NDF Patient Advocacy Program Manager

Advocates - \$500 to \$999

Matt Albert
S Azevedo
John Bambury
Jennifer Yashari & Noah Bartfeld
Arezou & Stephan Berghoff
Jessica Bowman
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Golden Doaba Enterprises
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Dana Feldstein
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Carol & Kevin Gelbard
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John Steven Gordon
Kacie and Adam Griffin
David Hakakian
Sylvia Hakim

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Cyrus Etemad
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Deborah Haroonian
Tina Hay
Bahman & Ruth Kamali
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Haleh Kamrava
Sepi Kashari
Ashley Khakshouri
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Ramin Matian
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Connie & Roger Bandera
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Laura Bass
Gail Shust & Joe Batutis
George Beast
Barbara Becher
Jackie & Gary Becher
Cassie & Michael Becher
Maryellen & Scott Becher
Rebecca Benhaghazar
Doris Benlevy
Julie & Justin Berger
Holly & Bob Binafard
Valentine & Bill Boyd
Erin Bray

I've been on the board of NDF Ambassadors for almost five years and it truly feels like we are a family. Since joining the organization, I have gained so much knowledge about the research and commitment being put into finding a cure that I felt it was important to be screened to spread awareness to my community. This collective energy inspires me to encourage peers to become conscious and assist in our efforts for treatment and prevention.

- Desiree Pakravan
NDF Ambassadors Co-Chair

Sharon Navidzadeh
Daniel Naysan
Emina Nazarian
Farahnaz & Homayom Nehoray
Tyler Neman
Nikou & David Norouzi
Shan Nourian
Stella Pae
Daniel Pantucci
Shane Parker
Ghazaleh (Rachel) Partiel
Varsha Patel
Jayesh Patel
Jeffrey Perry
Barrie & Adam Pivko
Perfect Properties
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Hava & Paul Rahimian
Douglas Ramsey
Mahesh Ranchhod
Jen Sack & Evan Rothenberg

William Rowe
Shahnaz & Hoshang Saghian
Candace & David Samadi
Jeannine & Nouzhan Sehati
Betty & Daniel Shalom
Claudine Shokouhi
Yosef Simsoly
Goly & Benny Soleimani
Caroline & Robby Soofer
Carlo Tabibi
John Van Duyn
Hagit Elul & Boaz Weinstein
Tracy & Sam Yadegar
Ty Yancey
Katrin & Rouben Youssian
Jimmy Yung
Nilou & Jack Zakariaie
Hiren Zaveri
Karen & Raymond Zolekhian
Lorne Zwaresh

Kimberly Mooney
Saba Moreh
Babak Mortazavi
Mahnaz & Bijan Nahai
Arjang Naminik
Nilou & Ariel Namvar
Melissa & Amanda Nassirzadeh
Ervin Nazarian
Atoosa & Alex Nehorai
Nihal LLC
Shahnaz & Abraham Ohebsion
O'Neil Omidvar
Sandra Pirnazari
Aram Pogosian
Mahnaz Pouratian
Nathaniel Roshodesh
Helen & Nejat Rostami MD
Shaw (Shahram) Saeedian
Ryan Saghian
Jamie Sands
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Golnar Setareh
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Ivan Wolkind
Rabbi David Wolpe
Martha Wright
Sam Yamini
Elena & Kouroshe Yeshoua
Leila Yousefzadeh
Shannon Larian & Jacob Zackary

NDF has gone from a fundraising organization that supported one or two research groups to an organization that is steering HIBM research. NDF is bringing together and directing top notch scientists who are now eager to work on a disease that they probably knew very little about 5-10 years ago. These scientists bring new enthusiasm, new ideas and passion to the cause.

In addition, the team has managed to seamlessly integrate science with patient advocacy and support by utilizing and incorporating the power of educated and dedicated patients.

- Dr. John Hakimi
Biotech Industry Expert
NDF Scientific Advisory Committee

Ross Cofer
 Benjamin Cohanazad
 Amy Cohen
 Stephanie & Matthew Cohen
 Gina & Paul Coletti
 Rebecca Conahan
 Richard Cooley
 Zoe & Daniel Corwin
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 Jennifer Cowan
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 Liora Simozar
 Nazila & Mehran Sina
 Deanna Sinfield
 Carla M Singleton
 Alison Skrinar
 Nicole & Josh Slavitt
 Sara & Larry Sloan
 Jenny Smiley
 Jiwon & Steven Song

NDF to me is a vehicle of hope, energy, and positivity, which would definitely be able to drive GNEM community through today's hardships to tomorrow's happiness.

- Ashutosh

GNEM patient and NDF Certified Patient Advocate, India

Vishal Patel
 Nima Pedram
 Casey Picard
 Sara Pitz
 Limor Pouldar
 Judy Price
 Amy & Navid Rachman
 Nilou Raiman
 Anjay Ranchhod
 Ryan Ranchhod
 Ajay Ranchod
 Candace Ravan
 Madeleine &
 Shahryar Ravanshenas
 Brian Reynolds
 Laura Richter
 Marc Riedl
 Elena & Jack Rochel
 Laner Rochelle
 Natasha Rojany
 Lauren & Gil Rojany
 Jodie & Micha Rojany
 Michelle & Marc Rosenbach
 Malancy & Mike Rosenzweig
 Farivar Roshanian
 Cyndi Sarnoff Ross &
 Michael Ross
 Tomer Rothschild
 Marilyn & Irwin Rudy
 Fred Rupnarain
 Mirriam Saeed
 Eliot Saeedi
 Nicole Sakhai
 Benjamin Samuels
 Julie Sandor
 Robert Sanger
 Laura Saul
 Ellissa Schneider
 Steven Seidman

Rachel Soroudi
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 Lori Zuckerman &
 Thomas Strouse
 Jane & Justin Suhr
 Rachel Sumekh
 Ronald Swan
 Catherine Sweeney
 Ghazall Tahour
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 Cynthia Tobisman
 Brigitte Toubia
 Herman Voogel
 Tara Voogel
 Steven Ware
 Denise & Larry Weintraub
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 Howard Weissman
 David Werner
 Mary Wheless
 David Wick
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 Don Williams
 Barbara Wuebbels
 Loris Yadegaran
 Wee Yan Loong
 Jennifer & Jake York
 Brandon Younesi
 Maryam Younesi
 Salim Youssefzadeh
 Jesse & Amit Zilberstein
 Andrea Zirman

* 2018 Pledge in Progress

** 2018 Pledge Pending

NDF Impact

NDF is the world's largest GNEM-only patient advocacy organization. Our programs fund scientific research and provide comprehensive resources, support and advocacy to affected individuals and their families/caregivers. NDF provides outreach and education to scientists and physicians who collaborate, share data to facilitate proper diagnosis, and encourage timely genetic testing to prevent the passing down of the disorder to future generations.

2019 Scientific Initiatives

Financial Support (in the form of a gift) of the National Human Genome Research Institute (NHGRI) of The National Institutes of Health (NIH) Phase 2b clinical trial entitled, "A Randomized, Double-Blinded, Placebo-Controlled, Multi-Center Study to Evaluate the Efficacy of N-acetyl-D-Mannosamine (ManNAc) in Subjects with GNE Myopathy.

Funding of Goldyne Savad Institute of Gene Therapy, Hadassah Hebrew University Medical Center's research in the development of a knock-in mouse model and a zebrafish study. If successful, these animal models will clear a long-standing obstacle in this area and will expedite approval at the FDA level.

Funding of Lek Lab, Department of Genetics at Yale University School of Medicine research to identify and develop new biomarkers useful in tracking therapies, including gene therapy. A second proposal, investigating a CRISPR system, may provide an alternative form of gene therapy which may be more useful in certain patients.

Funding of Mount Saint Joseph University research to use sialylation-sensitive lectins in GNEM patient myoblast cells with a variety of GNE mutations to identify biomarkers of disease, which can then be used to assess the potential of treatments such as gene therapy.

2019 Program Goals

Develop Patient Registry

A patient registry is the storage of many individuals' personal information and their medical history. It is typically disease-specific. Its compilation allows researchers to more accurately estimate both the prevalence and incidence of a disease and to determine the patients' natural history. Once that information is known, it can be used to more accurately advise patients and improve their care, which can lead to improved health and life expectancy — even in the absence of a cure. For this reason, patient registries are a key resource for biopharma companies to identify eligible patient populations for gene therapy research, trials and treatments. In 2019, NDF will focus on the compilation of a GNEM database, led for and by patients. The project will be supported by an NDF-sponsored internship and overseen by our scientific director, Dr. Monkol Lek, at Yale University.

Increase International Collaboration

In rare disease, international cooperation is vital to obtaining sufficient information, not only to compile patient registries but also to realize the full potential of all studies of the disease. Scientists can then build upon that data. NDF requires that all its grantees share their data — rather than work in independent silos. This is part of all our funding agreements. In 2019, NDF plans to continue its goal of serving as a unifying force among academic and research facilities worldwide. It is only through our collective efforts that progress can be made in quantifiable and meaningful ways.

Expedite Steps to Gene Therapy

Science takes time and certain processes cannot be rushed. However, new scientific contractors and advisors will be added to our roster of esteemed scientists, aiding us in pursuing our primary goal for 2019 — to continue providing the FDA with answers to their questions through studies; to identify, solicit and select a biotech partner that has the interest, (substantial) resources and professional inclination to take gene therapy to market. Financially, our goal is to raise \$3 million by December of 2019 in order to cover the initial expenses to phase 1. These funds will put us and our patients in a better position than many other rare disease patients who don't have a supporting organization successfully getting biotech focused on their disease.

Patient Advocacy

NDF will add cognitive well-being and mental health component to its advocacy program with the creation of a monthly, virtual support group for patients.

NDF'S 6th Annual Symposium on GNE Myopathy will spread its reach by taking place in several cities in 2019 including Philadelphia, Israel, Côte d'Ivoire, and Italy.

NDF will present an update at a GNEM patient day in India.

NDF's series of successful online seminars for patients will continue to provide scientific updates and topical subjects to patients and stakeholders, worldwide.

NDF will add a new class of Certified Patient Advocates to help empower patients to locally advocate for themselves and others.

Outreach and Education

NDF Ambassadors will host events to educate young adults about GNEM and to promote genetic screening in our efforts to prevent passing down the disease to future generations.

NDF will further its reach in the medical community with its inclusion in the 2019 Rare Neurological Disease Report with distribution to 20,000 neurologists in the United States.

NDF's United Youth will expand its reach to create a safe space for open dialog and advice among the children of GNEM patients.



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- 1 IRS "Tipping Law" mandates that no one entity can provide a majority of funding for charitable organizations. Therefore, our \$2.5M match grant was broken into two parts, and the \$1M received in 2018 was matched by our many individual generous donors listed in this document.
- 2 Officially called GNE Myopathy, commonly known as HIBM (Hereditary Inclusion Body Myopathy). Also known as: Nonaka Myopathy, DMRV (Distal Myopathy with Rimmed Vacuoles), QSM (Quadriceps Sparing Myopathy), HIBM2 (Hereditary Inclusion Body Myopathy Type 2), IBM2 (Inclusion Body Myopathy Type 2).
- 3 For more details see clinicaltrials.gov ID: NCT01784679 and NCT01417533.
- 4 Based on publicly shared IRS compliant financial statements. Please visit [guidestar.org](https://www.guidestar.org) or [curehibm.org](https://www.curehibm.org) for more financial details. 2019 Annual Report based on 2018 tax returns.