NEUROMUSCULAR
DISEASE FOUNDATION

Annual Report & Impact Statement

Review of the 2020 Calendar Year

Message from our CEO

Dear Supporters,

Lalé J. Welsh
CEO, Neuromuscular Disease Foundation

There is no question that the year 2020 will go down as one of the **most disruptive in recent history**. Through a global pandemic that accentuated health inequities everywhere, polarizing politics, and volatile financial pressures caused by business shut-downs, I am honored to report that **the Neuromuscular Disease Foundation, not only survived, but thrived working extra hard and rising to the challenge to deliver high-value resources, programming and service to all of our stakeholders, particularly for patients with compromised health issues.** And we did this within a year when our funding was greatly diminished.

With a modest PPP loan and the support of our most committed donors listed in this financial report (to whom I am especially grateful this year), we were able to raise $440K to stay afloat and thrive. This came at a time when many people reasonably expected NDF to implode like so many other for-profits and nonprofits alike. While thousands of U.S. businesses filed for bankruptcy, the explosion in the need for assistance from charities quickly outstripped available resources by a factor of 10[1].

COVID-19 did not shut us down. Quite the opposite - our ever-evolving work, not only remained consistent with our drive to fulfill NDF's critical mission, but it was carried out deftly and efficiently thanks to the dedication of our staff, Nancy Lurie and Rachel Leib, who sprang into action with me to implement new ways of presenting our patient programs, coupled with the wisdom of Board members who approved experimental new programs, amidst the chaos, in order to further serve our patient population.

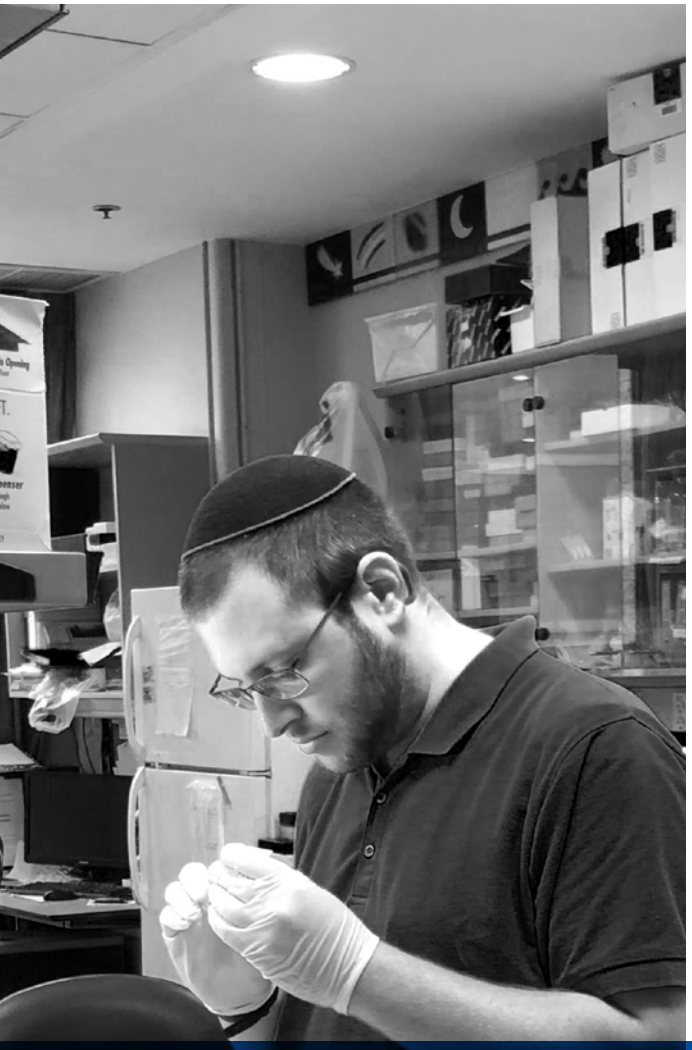
As examples, I present the following highlights of 2020:

- Design and approval of the world's **first multi-disciplinary, GNE Myopathy-specific clinics, free to our patients**. This collaboration with UC Irvine was modeled after the Muscular Dystrophy Association's clinics and is led by our own Board member, Dr. Tahseen Mozaffar, and his team of medical experts.
- $811K of funding towards **critical scientific research** that has addressed FDA feedback and has propelled us closer to approval for an IND. Human clinical trials are now expected in 2023, despite the pandemic.
- Launch of our live, weekly **Virtual Speaker Series** to update and educate the public in real time, which added over 40 hours of programming for "on demand downloads" from our Learning Library that has created an up-to-date treasure trove repository of GNE Myopathy scientific data for our scientific community.
- And professional invitations into the inner sanctum at Scribe Therapeutics, the well-funded biotech and brainchild of the celebrated **Dr. Jennifer Doudna; Nobel Prize winner and inventor of CRISPR technology**.
  I am honored to report that these meetings have resulted in one of our esteemed scientists being invited onto their Scientific Advisory Board, thus elevating the much-needed awareness of our rare disease into the purview of the scientific elite.

As always, none of this progress would be possible without the support of you — our generous donors. On behalf of the Board of Directors, scientific advisors, staff, and patients and their family members, I wish to thank each and every one of you for your past support. It is our great hope to see all of you at our upcoming gala in Los Angeles on November 14 (COVID-19 restrictions permitting). We can then celebrate the progress we have made so far and raise the necessary funds so desperately needed to bring our goals to fruition ahead of many other rare diseases.

P.S. Please take a few minutes to check out our latest accomplishment - the launch of our NEW WEBSITE – CureGNEM.org.

1 Washington Post, August 2020



About
GNE Myopathy

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

GNE Myopathy is a rare, inherited disease that causes progressive muscle weakness. The disease is caused by defects of the GNE gene. In this disorder the defect of the GNE enzyme disrupts the functioning of the sialic acid metabolic pathway, and interferes with normal muscle function.
GNE Myopathy typically affects young adults. One of the first signs of the disease includes inability to lift the front of the foot (foot drop). This may cause toes to drag while walking. As the disease progresses, it leads to various degrees of compromised mobility. GNE Myopathy does not affect the muscles of the face, or swallowing ability. It does not typically affect the heart or breathing muscles.

GNE Myopathy is a rare disease estimated to affect around 6 in 1,000,000 people. It affects populations worldwide but is more common in certain Asian, European and Jewish ethnicities. Like all rare diseases, GNE Myopathy is under-diagnosed with many people still pending proper diagnosis globally.

GNE Myopathy has also been known as HIBM (Hereditary Inclusion Body Myopathy), Nonaka Myopathy, Distal Myopathy with Rimmed Vacuoles, Inclusion Body Myopathy 2 (IBM 2), and Quadriceps-Sparing Myopathy. To facilitate diagnosis, GNE myopathy is the preferred name.

We are grateful to our international consortium of scientific GNE Myopathy experts for their collaboration with NDF and with each other, which continues to contribute invaluably to the expedition of our common goals and mission.

Scientific Advisory Committee

Dr. Zohar Argov
Dr. Carmen Bertoni
Dr. Kelly Crowe
Dr. Nuria Carrillo
Dr. Wayne Grody
Dr. John Hakimi
Dr. Angela Lek
Dr. Monkol Lek
Dr. Tahseen Mozaffar [B]
Dr. Stella Mitrani-Rosenbaum
Dr. Laura Rufibach
Dr. Perry Shieh

B - member of NDF Board of Directors

Scientific Advisors, Board members, and staff at our 2019 Gala

2020 Grant Recipients

Hadassah Medical Center (Israel)
Development of Animal Models

Dr. Stella Mitrani-Rosenbaum continued her work to develop mouse and zebrafish models lacking expression of GNE. Animal models are necessary to assess efficacy of any therapy and establish dose needed to achieve an effect. Furthermore, these models will enable us to stimulate the interest and gain the attention of pharmaceutical companies interested in developing and marketing a cure for HIBM.

Lek Lab at The Yale School of Medicine
Biobank

Drs. Angela and Monkol Lek continue their work establishing a repository of cells from patients, family members and eventually animal models as a resource for the GNE Myopathy community of scientists and pharmaceutical companies for research purposes (including identification of biomarkers and to validate therapeutic applications). To date, the Lek Lab was able to establish and bank 40 cell lines. All are available as fibroblasts (skin cells) as well as myoblasts (muscle cells). DNA and RNA have been collected and stored for upcoming sequencing and samples have been already shared with other investigators in the HIBM field. Future plans include further collection and storage of samples that contain different mutations so as to obtain a good representation of all HIBM patients worldwide.

> Thanks for all the work you do on behalf of GNEM patients. I am grateful and know you work diligently to find us a cure.
>
> **- Tara, GNE Myopathy patient**

Lek Lab at The Yale School of Medicine
CRISPR Gene Editing Approaches

Dr. Angela Lek focused on conducting proof-of-concept studies in cells in vitro and at correcting the M743T mutation in patient fibroblasts. The cell lines and constructs needed to carry on the project have been successfully generated and results on the feasibility of using CRISPR-mediated gene editing approaches should be obtained.

Lek Lab at Yale School of Medicine & PerkinElmer Whole Genomic Sequencing

Our whole genomic sequencing project continues in our effort to help identify any modifiers and allows the medical community to better understand the reasons that patients show such diversity in the progression of the disease. We have collated the second wave of samples for WGS, which included critical samples collated from Iran and other samples collected from the various NDF patient conferences.[1]

Mount St. Joseph University Lectin Staining Biomarkers

Dr. Kelly Crowe tested levels of GNE Myopathy biomarkers in patient cells in vitro before and following gene therapy. Studies will then be extended to patient muscle biopsies as well as zebrafish models for the disease. The results obtained are particularly important because, if successful, they will enable us to establish outcome measures to be used in clinical trials and are required by the FDA in order to receive approval of the therapeutic approach being tested.

National Center of Neurology and Psychiatry (Japan) Activation of Endogenous Mutated GNE Product by Small Compound

Dr. Ichizo Nishino and Dr. Wakako Yoshioka undertook a drug discovery project aimed at identifying compounds capable of activating the GNE/MNK kinase. The goal is to identify drugs that activate GNE/MNK kinase and therefore elevate supply of sialic acid (SA) to the whole body, including muscles. If proven safe and effective, the drug could be used alone or in combination with gene therapy- mediated approaches to HIBM, thus increasing the efficacy of the clinical approach.

National Institutes of Health (NIH) Identification of Biomarkers

Dr. Marjan Huizing focused on identifying biomarkers and gene modifiers that could play a role in disease progression in order to identify new players in the disease as well as new potential outcome measures to use in clinical trials to validate the efficacy of therapy. Studies focus on identifying additional biomarkers which require less invasive procedures than muscle biopsy.

> Your focus on maximizing funds to go to towards funding research, education & patient care is remarkable. Thank you so so much!!!!
>
> **- Kevin, GNE Myopathy patient**

Nationwide Children's Hospital Development of FDA-Compliant Gene Therapy Assays for GNE Myopathy

Dr. Paul Martin focused on developing a gene therapy clinical development plan to be used to demonstrate activity of AAV-mediated delivery of GNE into cells and muscles of mice. In particular, the project will establish a potency assay that effectively describes the biological activity of the AAV vector to be used, in this case an AAV.GNE gene therapy vector.

The Ohio State University Compromised Membrane Repair

Dr. Noah Weisleder suggests that muscle cells isolated from GNE myopathy patients show to be more susceptible to damage than cells isolated from non-affected individuals. This finding is particularly important because it could explain the slow deterioration of muscles in patients. Furthermore, these studies will help establish important biomarkers and outcome measures to be used in future clinical trials which are critically needed at this stage of clinical development for HIBM. The assays being developed in the Weisleider Lab could, in fact, become an integral part of clinical practices in human trials and to identify different stages of the disease. In addition, the assay could be used to prove the efficacy of the gene therapy application being tested, thus becoming a valuable tool to ensure FDA approval.

1 Update: The sequencing was performed in early 2021 and the analysis by the Lek Lab is currently on-going. Future plans for this year will involve collating the final wave of sequencing to complete the targeted 100 WGS samples.

Highlights
By the Numbers

93% of diseases

have no FDA approved therapies or cures.

There are 7000 known rare diseases affecting 1 in 10 Americans. More than 30 million Americans are living with a rare disease.

GNE Myopathy is one of ~5000 rare, monogenic diseases potentially eligible for gene therapy.

64%

of our income in 2020 came from grants. We are grateful to have been awarded grants by: The EveryLife Foundation for Rare Diseases, The Confidence Foundation, Whittier Trust, The Rotary Club of Beverly Hills, and The Iranian American Jewish Federation - New York.

79 cents of every dollar

goes towards funding scientific research and core programs providing critical support to families living with GNE Myopathy.*

* vs. US-based non-rofit organization average of 63%

100% of programming

In spite of a global pandemic and compared to the previous year, we provided 100% of our programming with only 16% of the income.

> I'd like to thank you so so much for the work that you're doing! Sometimes this particular "walk" can be a lonely one for both the patient and their loved ones.
>
> **- Parent of a GNE Myopathy patient**

+18% patients

Our list of known patients grew by 18% and our overall contact list grew by 5% in 2020 – mostly attributable to our Speaker Series.

22% of all new contacts were GNE Myopathy patients.

1531 people

are served annually by our programs.

$7,300,000 funded

to date, in service of GNE Myopathy programs.

2020 Income

Grants	64 %
Donations	24 %
Gala Pledge Payments	7 %
Earned Income	4 %
Events	1 %

2020 Expenses

Clinical Research	59 %
Patient Advocacy	8 %
Education, Awareness & Outreach	11 %
Clinics	1 %
Admin & Professional Services	11 %
Fundraising	10 %

Outreach & Education

Symposium Speaker Series

In 2020, in response to the COVID-19 pandemic, NDF quickly reinvented its in-person Symposia to a weekly, online Symposium Speaker Series featuring scientists and experts in many fields of interest to our patients, which has served to maintain critical discourse among scientists, educate the patient community and actually expanded our reach. Each week an expert in their field made a presentation about their NDF funded study or field of expertise, moderated by another scientist or NDF team member.

After each presentation, attendees (fellow scientists, industry professionals, family members and patients) were able to have their questions answered, creating a unique opportunity for interaction among community members.

Our online Learning Library has created an invaluable resource for visitors of our website to watch recordings of each presentation.

5
Months

22
Presenters

500+
Live Attendees

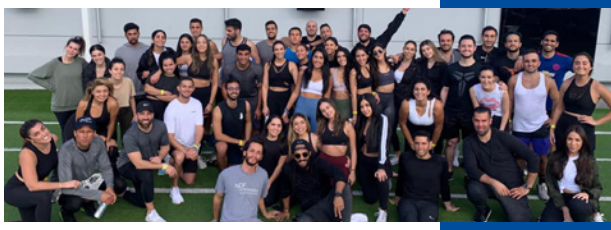
2000
Library Views

Genetic Screening Education

As a member of the Jewish Genetic Disease Consortium and in partnership with JScreen, we kicked off 2020 with in person programs which introduced clergy members and members of the Los Angeles community to the importance of pre-conception genetic screening and disease prevention, which were featured in **an article published in the Jewish Journal** with a circulation of 150,000 weekly readers.

NDF's Ambassadors, our committee of young professional adults in Los Angeles, worked with us and our partners at JScreen to create a short, **educational video about GNE Myopathy** and the importance of pre-conception genetic screening.

Educational materials were distributed via socially distant campaigns targeted at newlyweds and those thinking about family planning.



In January 2020, **NDF Ambassadors hosted a fun, physical event** to raise awareness for NDF and GNE Myopathy among the young adult community in Los Angeles.

Education & Awareness Event in West Africa

Organized by NDF Certified Patient Advocate, Rose Okoma, and sponsored by NDF, this meeting in early 2020 sought to educate doctors and scientists in West Africa about the challenges of diagnosis and management of GNE Myopathy and other rare neuromuscular diseases in the face of therapeutic advances in the rest of the world. Doctors were also on-hand to speak to patients from the region who attended and who have struggled to be properly diagnosed.

Clinics

NDF-Sponsored, Multi-Disciplinary Clinic for GNE Myopathy Patients

Dr. Tahseen Mozaffar

To address the GNE Myopathy community's physical wellness while they wait for treatments and a cure, the NDF Board, in association with The University of California, Irvine's Neuromuscular Center and under the direction of GNE Myopathy expert and NDF Board of Directors and Scientific Advisory Committee member Dr. Tahseen Mozaffar, approved the launch of a first-of-its-kind **GNE Myopathy Multi-Disciplinary Clinic**. The creation of a clinic available to patients, either in person or via telehealth, and specific to the needs of GNE Myopathy patients has become necessary in ways never imagined before. Currently, because of their disparate geographical locations, our patient population is limited to physicians and healthcare providers who may not fully understand GNE Myopathy, if at all, and may therefore not be in a position to make accurate judgments about secondary health issues. Being able to provide one-on-one health care by medical professionals trained in GNE Myopathy ensures that patients get optimal diagnostic procedures and receive state-of-the-art treatment at no cost to them. Patients at the clinic will be seen by specialists well versed in GNE Myopathy that include neurologists, physical therapists, occupational therapists, social workers, and geneticists.

Holistic Care

Carol Gelbard, LCSW

In 2020 the emotional needs of our patient community became more apparent than ever before. In order to better meet the needs of people living with GNE Myopathy and their family members, NDF added Carol Gelbard, LCSW to our team as our dedicated **Emotional Wellness Director**.

Carol has many years of experience with GNE Myopathy patients, is an advocate for counseling and has been able to help us advance many of our patient programs.

With our **Emotional Wellness Clinic**, NDF offers GNE Myopathy patients an evaluation and needs assessment session with our Emotional Wellness Director at no cost to them. These sessions provide a mental health "check in" to help determine one's current needs in an effort to formulate recommendations for further supportive services. The consultation may be held via telephone or video session.

> I have been feeling very emotional and upset lately… Carol is brilliant. I have never really had any form of counselling like this, so it was really valued. She was able to understand me and help me support myself better. **Thank you so much for giving me that space to be open and honest.**
>
> **- Anonymous GNE Myopathy Patient**

Support

Programs & Groups

HUDDLEs

Our monthly, virtual, peer-to-peer patient support meetings, called **HUDDLEs**, are confidential; giving patients an opportunity to discuss any topic in an open, safe and non-judgmental forum. People diagnosed with GNE Myopathy from all over the world join together monthly to discuss their shared experiences. Conversations about specific topics of interest and choice are often moderated by different patients, providing leadership opportunities and differing points of view.

NDF Talks

NDF Talks is our Podcast series launched in 2020 and available on our website, on demand and on most podcast streaming services. Each episode provides insight into the experience of a member of the GNE Myopathy community. Scientists, patients, caregivers, therapists and industry professionals are asked questions of particular interest to our listeners in our effort to provide content that is engaging and specifically relevant to the GNE Myopathy community.

Virtual Hang Outs

NDF Virtual Hang Outs, debuted in 2020, are purely social events for patients. Launched with a COVID-19 friendly, virtual game night, this event allowed patients from different countries to be together, doing something fun, while at home because of COVID-19 restrictions. This event was so popular that we intend to repeat it regularly, post pandemic, in an effort to allow patients the opportunity to continue gathering virtually.

> Being new to GNEM means that I am still processing and coping with the difficulties that have changed my life. I joined this HUDDLE to hear what others had to say about how they feel and be heard in a place where I know I'm understood. It was wonderful to be able to express those feelings without feeling judged or ashamed. The conversation was varied and the information and support provided was very helpful! I am so thankful for this support system!
>
> **- Sidney, GNE Myopathy patient**

United Youth

NDF United Youth is a group dedicated to supporting the children and young carers of our patients. Their private Facebook page offers a safe space for children of people with neuromuscular disease (not limited to GNE Myopathy) to share their experiences and offer advice to one another.

Ambassadors

NDF Ambassadors is a group of motivated, young professionals focused on raising awareness about GNE Myopathy and educating people about the disease and the importance and value of pre-conception genetic screening.

Caregivers

Our **Care Partner Support Group** is designed to be a supportive and safe place for caregivers, care partners and family members who have a GNE Myopathy patient in their lives to support each other and talk about their shared experiences. Programming is created with their specific needs in mind.

Support

Certified Patient Advocates

Our **Certified Patient Advocate Program** empowers GNE Myopathy patients worldwide to work as our partners in advocating for and creating programming in their home countries.

In 2020 we grew our Certified Patient Advocate Program by 50%.

Our goal is to have Certified Patient Advocates in every country where we have identified patients.

> Every day is extremely challenging for me in so many ways I never imagined for myself at this stage of my life. Nevertheless, I have found more creative ways of adapting. I persevere and find a way to get myself where I need to be. As a fellow GNEM-er once told me, 'We still have full use of our mouth and brain," which has proven more useful than the use of limbs in certain scenarios! It's important to remember that we can live a "normal" life like everyone else. I am managing my disability through my mindset. Where there is a will, there is a way.
>
> **- Valenee, GNE Myopathy Patient and NDF Certified Patient Advocate**

In 2020 we worked with our Certified Patient Advocates in Turkey, the Ivory Coast, and the UK to host events for the patient communities in their countries. Certified Patient Advocates in multiple countries attended events (in person at the beginning of the year and virtually, later on) representing NDF and our mission to educate people about our work and their disease.

United States:

- Melissa Branch – Southeast
- Amy Curran – East
- Imad Kazim – New England
- Tara Voogel – West

Africa:

- Rose Okoma – Ivory Coast

Asia:

- Sabeen Faisal – Pakistan
- Tae-Eun Kang – Korea
- Kelly Ma – Taiwan/China
- Ashutosh Verma – India

Europe:

- Abajee Dharmajee – France
- Valenee Gosine – U.K. South
- Saskia Melches – Germany
- Vaneesha Mohadawo – France
- Michela Onali – Italy
- Valeria Pace – Italy
- Mona Patel – U.K. North

Middle East:

- Roberta Cirisyan – Turkey/Greece
- Maya Davidovich Cohen – Israel
- Suleyman Kus – Turkey
- Guy Ratson – Israel
- Abdullah Al Shamrani – Saudi Arabia

Support
Awards

While weren't able to acknowledge our honorees in person at a gala in 2020, we were able to highlight the consequential contributions of our 2020 award recipients: Founders' Circle Award winner NDF Founder, Gila Michael, on whose shoulders we stand today; Advocacy Award winner Michael Banayan; and Outstanding Service Award winner Doran Adhami, who both completed their long service to the Board of Directors in 2020.

Our 2020 awardees will be recognized, along with our 2021 winners, at our 2021 Gala.


Gila Michael


Michael Banayan


Doran Adhami

Patient Fundraising Challenge Winners

NDF All-Stars

NDF's 2020 Allstar Campaign was a coordinated effort by GNE Myopathy patients to raise funds and awareness for NDF as a team. We are very grateful to the patients around the world who, by participating, became proactive members of our fundraising team.

Special thanks to our winners: Imad for raising the most dollars, Valenee for bringing the most new donors to our cause, and special mention goes to Jennifer for her enduring and consistent support of our programmatic and fundraising efforts.


Imad

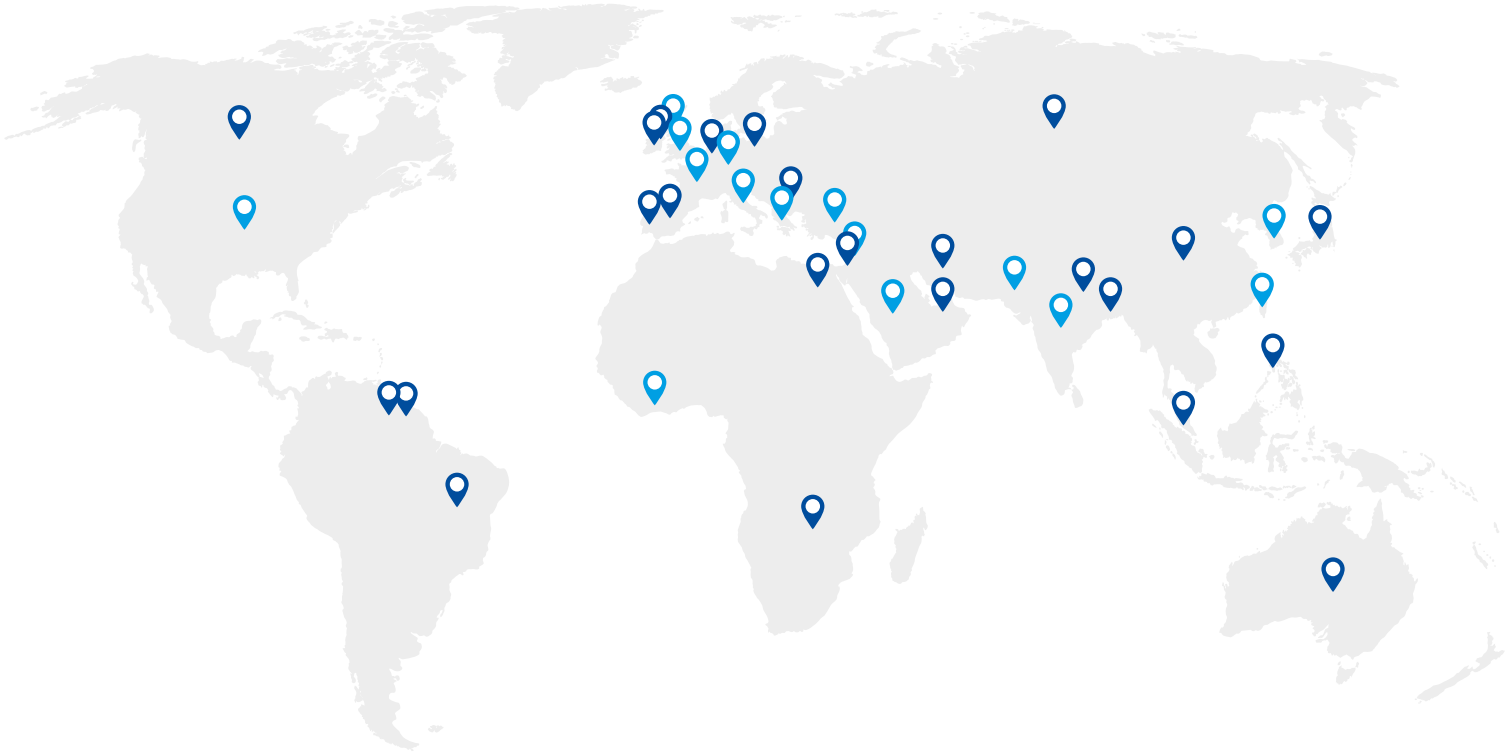

Valenee


Jennifer

> NDF has been aggressive in its quest in pursuit of treatments and gene therapy for GNEM. Most of the 7000 orphan diseases do not have any approved treatments, and NDF is way ahead of the game.

- Dr. Tahseen Mozaffar, NDF Clinic Lead
Member, Board of Directors; Member, Scientific Advisory Committee

Support
Global Impact



39 Countries & Territories

local Certified Patient Advocate

- Australia
- Bangladesh
- Brazil
- Bulgaria
- Canada
- China
- Egypt
- England
- France
- Germany
- Greece
- Guyana
- India
- Iran
- Ireland
- Israel
- Italy
- Ivory Coast
- Japan
- Malaysia
- Nepal
- The Netherlands
- Northern Ireland
- Pakistan
- Palestinian Territories
- Philippines
- Poland
- Portugal
- Russia
- Saudi Arabia
- Scotland
- Spain
- South Korea
- Suriname
- Taiwan
- Turkey
- United Arab Emirates
- United States
- Zambia

Thank you

Your support makes our work possible

Visionaries - $100,000 and up

Confidence Foundation
Iranian American Jewish Federation of New York

Benefactors - $25,000 - $99,999

Whittier Trust

Heroes - $15,000 - $24,999

Carolyn Yashari Becher and Rob Becher
Nasser David Haverim (WUO)
Lalé Welsh

Champions - $10,000 - $14,999

Maria Montgomery

Leaders - $5,000 - $9,999

Rotary Club of Beverly Hills
Orna Ravaei-Delrahim
Taban Family

Partners - $2,500 - $4,999

Emser International, LLC
EveryLife Foundation
Ralph Loren
Sepi & Robin Nourmand
David Yerushalmi

Builders - $1,000 - $2,499

Wonderful Giving
Neurology Reviews
The Raich Family Fund
M. Asselin - J Meltzer Fund
Tawnie Crowe
Jeanne & Carl Faber
Martha Kadue
Imad Kazim
Tricia Mullins
Nancy & Robert Lurie
Michele Ovesey
Stacy & Jesse Sharf
Lida & Ebrahim Simhaee
Jila & Benjamin Yadegar

Advocates - $500 - $999

American Online Giving Foundation
Elaine Albert
Elijah Aron
Paul Gilmour
Javed Khan
Jennifer Yashari

Supporters - $250 - $499

Network for Good
Amy Bersch and Christopher Crain
Nuria Carrillo
Jay Dee
Wayne Grody
Sue & John Hakimi
Tahseen Mozaffar
Becca Pitman
Angela Shoushani
Mayumi Suzuki
Jennifer Wilk

Friends - Under $250

Barbara Adler
Paulina Afshani
Gerard Aghai
Farad Ahmed
Rebecca Ainley
Matt Albert
Julie Alexander
Stella Altadonna
Trina Ankney
Donna Araneo
Shahab Arianpour
Kathy Artman
Jennifer Babcock
Jason Baker
Lorraine Bakin
Danielle Baldassari
Lorraine Baldassari
Mary Baldassari-Price
Isaac Banafsheha
Maria Barr
Susan Barrett-Vukovich
Dawn Bassano
Jackie & Gary Becher
Gopi Bhansali
Barbara Bleimann
Thomas Bonica
Brian Boylan
Ginger Brandhorst
Chris Brick
Daniel Britts
Scott Brothers
Sophie Brown
Betsy Brown Braun
Alan Buckley
Alain Caillé
Debbie Cameron
Leigh Canning
Tom Carey
Ligia-Ioana Caruntu
Cinda Cason
Samantha Castano
Louise Cataldo
Gary Chartier
Janet Coiteux
Zoe & Danny Corwin
Rosetta Crupi
Michele Crupi
Fabian Cruz
Laina Curran
Jay Dassrath
Kolby Dauler
Ana David
Chandler Davis
Terri Davis
Ryan Dean
Kim Defenderfer
Abram Delecki
Candy Delorenzo
Lisa DeMattia
Elisha St. Denis
Eileen Dershowitz
Vid Dhanraj
Gayle Diamond
Brian Dige
Eric Dilks
Steven Dollar
Emily Dossett & Giancarlo DiMassa
Sheel Douglas
Jon Drucker
Sangeeta Dubb
Lindsey Duran
Karin Edlund
Susan Elizabeth
Lauren Elizabeth
Hagit Elul
Patti Engel
Lili Eshaghzadeh
Justin Farahi
Ellen Felber
Kayla Fenner
David Ferrell
Amy Foster
Kim Fox
Irene Frances
Becca & Gary Frank
Jesica Gabayan
Rosaclara Gable
Pennie Gable
Eduardo Gamez
Mimi Ghitiu
Michelle Goldberg-Contovasilis
Merry Goldfarb
Shawn Golshani
Jo-Ann Gosine
Romana Gosine
Valenee Gosine
Leanna Grace
Bob Gray
Flora Habibian
Gali Hagel
Shane Hakakian
Bodil Hanell
Ronald Izzo
Brianna Jackson
Vijay Jagassar
Terry Janickey
Jadine Kachan
Erin Kelliher
Hooman Khalili
Kevin Kirby
Gilda Kohan
Ashley Kohanoff
Davina Krakovic-Patel
Martha Kravchak
Sourabh Kulkarni
Lesley Laine
Rochelle & Harvey Laner
Charles Larue
Emily Lefkowitz
Rachel Leib

> Being a parent of two patients of GNE Myopathy, one since 1998, I consider any donation, big or small, would be of immense help in finding a cure for this rare disease, so that in future no parent may see their child suffering from it. May the efforts of NDF see the light of the day.
>
> It is always a pleasure to interact with people who are doing so much for this noble cause. Keep up the good work.
>
> **- Javed, parent of GNE Myopathy patients and monthly donor**

Tammy Hardman
Alan Hardman
Yvonne Harris
Brenda Harrison
Shahrzad Haverim
Earl Hebert
Jonathan Hekmat
Bert Herrera
David Howard
Melissa Hunter
Michelle Hunter
Paulina Illoulian
Ross Iosefson
Nilo Irani
Caroline Irwin George
Susan Leib
Renee Leib
Myra Leib
Diane Leung
Mariel Levine
Robert Levy
Ian Ley
Nitara Lockwood
Jennifer Lubold
Kerstin Lundgren
Chloe Makhani
Karen Malatesta
Maxine Malekmehr
Deepak Malhotra
Navid Mansourian

Karen, Leigh and Abby Manuel
Jan Marie
Lindsay Marie
Daniel Markowitz
Megan Martin
Ann Mccabe
Jay Mccarthy
Sandy Mckinnon
Lisa Mclean-Peaston
Priscilla Mcwhorter
Simin Mel
Jennifer Melecio
Zelda Miller
Shawn Moradian
Mer Morrison
Ali Mortazavi
LeGrande Moseley
Deborah Mullins
Sharareh Najafi
Afsaneh Najafi
Dina Nazarian
Daniel Newman
Danny Nia
Vince Nichol
Gösta Nilsson
Rosemary Olivari
Larry Olivari
Steven Olivari
Sharon Olivari
Karen Olney
Leong Onn
Karin Östling
Anna Palmqvist
Mats Palmqvist
Kamalpreet Panesar
Zachary Parola
Lisa Parsons
Negar Partiali
John Partridge
Sarasvati Patel
David Pearce
Laura Pellizzi
Steve Pilot
Susan Powderly
Erin Puma
Chris Puma
Bejan Rafii
Andrew Rajpaulsingh
Guy Ratson
Farah Ravon
Patrick Redmond
Leeron Regev
Mary Reilly
Peter Ricciardi
Robert Rogers
Shannon Rourke
Shannon Rowland
Irwin Rudy
Angelina Russell
Jesse Salcedo
Brian Sandford
Leigh Sanzaro
Nahal Sarshar
Osamu Sasaki
Aaron Sassounian
Christopher Scott
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Tamar Shiloach
Ethan Shirian
Barbara Silverberg
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Liz Singh-Juarez
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