NEUROMUSCULAR DISEASE FOUNDATION

JOIN THE GLOBAL EFFORT TO CURE NEUROMUSCULAR DISEASES

2018 Annual Report & Impact Statement

Review of the 2017 Calendar Year

2017 was a year of palpable growth for NDF that dovetailed and solidified our many years of collective efforts. We experienced progress in all of our ongoing programs including our efforts toward gene therapy, our funding of a critical component of NIH's ManNAc trials, and the gathering of global scientific leaders and patients from 15 countries at our annual Symposium to become the world's leading GNEM advocacy group.

Growth and progress come at a cost, especially at the precipice of radical change that technology brings in terms of a potential cure. We are thankful for our GNE Myopathy community and their generosity to NDF. Their continued support allows NDF to make great strides year after year — funding all of the important work that we have begun, including, in particular, the funding of our next step towards gene therapy, which will require raising a staggering estimate of $5 million to continue the project beyond FDA approval.

Please continue to support NDF by donating, attending events and volunteering! Families living with GNE Myopathy have benefited immensely from NDF funding and we are so grateful for the commitment, resources and support.

Sincerely,

Lalé J. Welsh, CEO/Executive Director, NDF

NDF'S GLOBAL REACH

NDF's Certified Patient Advocacy (CPA) Program has gone international. Our CPAs (currently representing NDF in the US, Canada, United Kingdom, Turkey, Saudi Arabia, Italy, and Israel) have been hard at work educating people all over the world about GNEM.

September 2017—GNEM patients from 5 different regions in Italy gathered along with their family members with psychologists, sociologists, neurologists and physiotherapists for a day of education.

November 2017—Nine patients, 32 caregivers, and eight doctors/industry professionals attended the GNEM Meeting in Israel.

" Thank you NDF for empowering us to be proud of who we are as individuals and not for what affects us! Together, we are stronger. Proud to grow older with NDF."
— **Imad**, Boston, MA

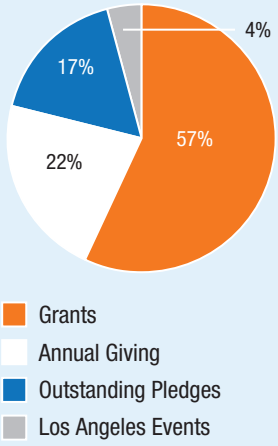
" NDF is keeping the hope alive for GNE Myopathy treatment."
— **Abdullah**, Riyadh, Saudi Arabia

" The Neuromuscular Disease Foundation gives me the possibility of not feeling alone anymore, of learning from those who fight my own battle and to be part of a community where everyone is valued for what, simply and wonderfully, they are."
— **Valeria**, Catania, Italy

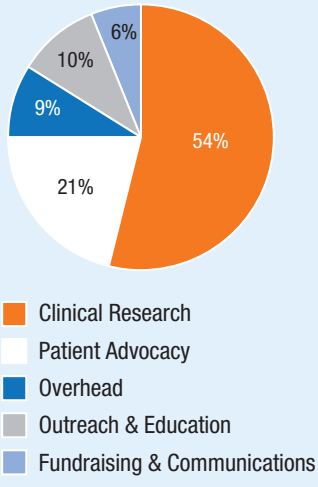
YOUR DONATIONS AT WORK

Over 90 cents† 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



Visit **CureHIBM.org** to learn more.

† Based on publicly shared IRS compliant financial statements

Please visit guidestar.org or curehibm.org for more financial details. 2018 Annual Report based on 2017 tax returns.

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.

2018 SCIENTIFIC INITIATIVES

- **Funding Hadassah Medical Center's research** in the identification of molecular pathways involved in the pathophysiology of GNE Myopathy and in potential protective mechanisms.
- **Financial support of the National Human Genome Research Institute (NHGRI), National Institute of Health's Phase 2b clinical trial** titled, "A Randomized, Double-Blinded, Placebo-Controlled, Multi-Center Study to Evaluate the Efficacy of N-acetyal-D-Mannosamine (ManNAc) in Subjects with GNE Myopathy."
- **Collaboration with PerkinElmer for Whole Genome Sequencing** (WGS) paired with metabolomics for analysis of 100 samples, in an effort to help us better understand the molecular pathways for GNE Myopathy, lead to finding effective therapies for GNEM and support our ongoing gene therapy research initiative.
- **Our Pre-IND agreement with Dr. Mendell is in its final phase** and we are gearing up for the next step—a toxicology study (tested on mammals prior to human dosing). This next step is estimated to cost close to $500,000, and we expect that human dosing can begin 12–18 months from its start date in 2018.
- **Advocate for additional gene therapy funding estimated at $25 million** with third party bio-tech firms, high net worth stakeholders, and non-governmental grant makers.

2018 PROGRAM GOALS

- **NDF is continuing to spearhead a formal consortium of key researchers** who have agreed to collaborate, convene annually, (this year at the ENMC Conference in Europe) and share data through a single database to expedite treatments including Gene Therapy. Members of NDF's scientific collaboration include: *Ultragenyx Pharmaceuticals, PerkinElmer, The Open Medicine Institute, Emory University, UCLA, UC Irvine, JScreen, The Jain Foundation, Yale University, Nationwide Children's Hospital, and the National Institutes of Health (NIH).*
- **NDF continues to add patients and data to its international patient registry** to ensure that the community is organized and trial-ready and to collect data needed for better disease understanding.
- **2018 Los Angeles Symposium** will be a patient-centric event to focus on advocacy, public education (outreach), genetic screenings, practical solutions for our patient population and large focus on Gene Therapy.
- **NDF Certified Patient Advocate Program empowers GNEM patients** to self-advocate through NDF sponsored patient days worldwide to reach patients unable to travel to our symposium.
- **NDF provides the latest data and resources to our international support group network** which consists of sister organizations in several countries, many of whom do not have the resources to form legal entities or access to such information.
- **NDF's Ambassadors Program continues to lead the effort to promote timely genetic testing** and steps to prevent passing down the disease to future generations, in addition to hosting friend and fundraising events in Los Angeles.
- **NDF kicks off its first ever series of on-line seminars for** the GNEM community featuring speakers and topics of interest for patients, family members and caregivers, worldwide.

* 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).

† For more details see clinicaltrials.gov ID: NCT01784679 and NCT01417533.

NDF MISSION

Our mission is to enhance the quality of the lives of people living with GNE Myopathy (also known as HIBM)* through advocacy, education, outreach and funding 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, world wide; however, ongoing natural history studies† show elevated carrier rates in certain populations of Eastern European and Asian heritage; including Jewish, Persian, Uzbeki, Arab, East Indian, Indonesian and Japanese to name a few.

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