NEUROMUSCULAR DISEASE FOUNDATION
JOIN THE GLOBAL EFFORT TO CURE NEUROMUSCULAR DISEASES

2017 Annual Report & Impact Statement

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.

NDF IMPACT

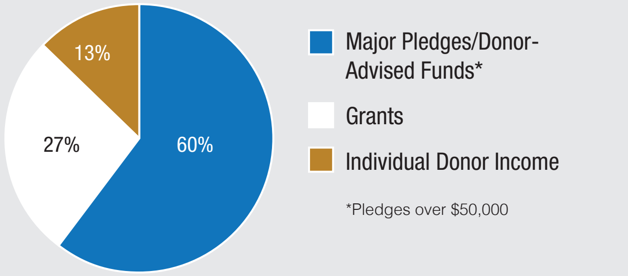
NDF is the world's largest GNEM-only patient advocacy organization. Our programs fund scientific research and provide comprehensive resources, support and advocacy to affected individuals and their families/caregivers. NDF provides outreach and education to scientists and physicians who collaborate,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

YOUR DONATIONS AT WORK

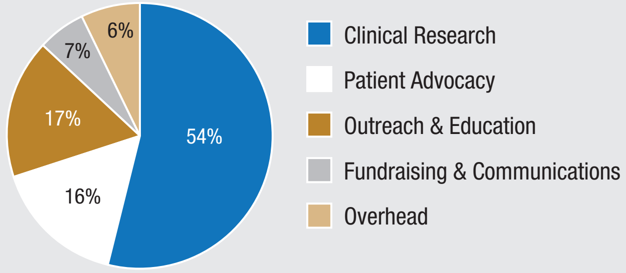
Over 90 cents[†] of every dollar donated goes straight to funding research and programs providing resources to families living with GNE Myopathy.

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

INCOME SOURCES



WHERE YOUR MONEY GOES



Please visit guidestar.org or curehibm.org for more financial details.
2017 Annual Report based on 2016 tax returns

OUR OUTREACH & ADVOCACY IMPACT

- **Over 1200 GNE Myopathy patients** and family members worldwide are served by our support programs and resources every year.[‡]
- NDF hosts **the largest annual symposium on GNE Myopathy in the world**, delivering education and support programs and connecting stakeholders, physicians, scientists and researchers with community members.
- We are **improving the quality of life** for affected individuals and their families and caretakers by facilitating social and networking events, support groups, health, nutrition and exercise workshops, as well as practical solutions to daily struggles for those living with disabilities related to GNEM.
- NDF's Ambassadors Program leads the effort to **promote timely genetic testing** and steps to prevent passing down the disease to future generations.
- Our Certified Patient Advocacy 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.

OUR SCIENTIFIC IMPACT

- For the past 10 years, NDF has **funded over $1.3 million in clinical research globally**, resulting in greater interest from principal investigators and industry alike.
- NDF is spearheading a **formal consortium of key researchers** who have agreed to collaborate, convene annually, and share data through a single database to expedite a cure. Members of NDF's scientific collaboration include: *Ultragenyx Pharmaceuticals, PerkinElmer, The Open Medicine Institute, Emory University, UCLA, UC Irvine, JScreen, The Jain Foundation, Ohio State University, Nationwide Children's Hospital,* and *the National Institutes of Health (NIH).*
- NDF maintains one of the **largest GNEM patient registries** in the world to ensure that the community is organized and trial-ready and to collect data needed for better disease understanding.
- NDF encourages patients to register for **natural history studies**.
- NDF's commitment to the **intersection of technology and biology** has led to the launch of an investigation with the FDA for gene therapy.
- NDF advocates for additional **gene therapy** funding estimated at $25 million with third party bio-tech firms, high net worth stakeholders, and non-governmental grant makers.

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[*] Officially called GNE Myopathy, commonly known as HIBM. 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).

[†] Based on publicly shared IRS compliant financial statements

[‡] Includes patients we reach through our symposium, online events, webinars, videos and patient days in and around the US, UK, Israel and India.

DONORS, SUPPORTERS AND PARTNERS:

GRANTORS & PARTNERS

Ultragenyx Pharmaceuticals
The Stanley & Joyce Black Family Foundation
Anonymous Donor Trust*
Global Genes
Emory University
Hadassah Medical Center
The Jain Foundation
JScreen
National Institutes of Health
Newcastle University
Open Medicine Institute
PerkinElmer
Rotary Club of Beverly Hills*
Treat-NMD
UCLA
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Natalie & Sohrab Shooshani*
Sharona & Afshin Veiseh*
Lalé Welsh*

* Donors from 2015 and 2016

> *“ NDF has not only raised awareness about HIBM, but has played a central role in bringing researchers of diverse background from all over the world together to collectively tackle this devastating disease. NDF has also promoted greater communication between researchers, patients and patient advocates, bringing a sense of community to those who suffer from the disease and the people who care about finding a cure.”*
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> — **SIAVASH KURDISTANI, MD**, PROFESSOR AND CHAIR, UCLA DEPARTMENT OF BIOLOGICAL CHEMISTRY

> *“ I am thankful for NDF’s help in eliminating the isolation felt by patients and for providing vital information to us and our caregivers. Their website and events are relevant and engaging. Most importantly, NDF empowers patients and carers to keep driving for change and to never give up hope.”*
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> — **MONA PATEL**, GNEM PATIENT, MANCHESTER, ENGLAND

> *“ Not every patient has an organization like NDF behind them, fighting for them, bringing patients together as a community, making us feel like a family, and truly believing we are the heart of the organization. With NDF, I know I am not alone and I have the greatest opportunity a patient could ask for when I participate in all that NDF has to offer.”*
>
> — **AMY CURRAN**, GNEM PATIENT, PHILADELPHIA, PENNSYLVANIA

> *“ It is invigorating seeing that NDF tirelessly works towards a cure and is a constant advocate for the GNE Myopathy patients throughout the year, every year. Thank you NDF for never giving up on reaching for a cure and being a champion for GNE Myopathy patients around the world”*
>
> — **DEBBIE SCOTT**, GNEM PATIENT, IRVINE, CALIFORNIA

ABOUT GNE MYOPATHY

GNE Myopathy — or HIBM — is a distal myopathy; a rare genetic disease starting at the feet, causing muscles to slowly weaken. HIBM 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. HIBM 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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[†] For more details see clinicaltrials.gov ID: NCT01784679 and NCT01417533.