FAQs

What's going on with the MONEY?

1. NDF staff have earned an unprecedented $2.5 Million grant from a private, U.S. based philanthropic organization (not a GNE myopathy stakeholder of any kind)
2. This amount needs to be matched in order to comply with IRS "Tipping laws" or it will have to be returned.
3. A check for $1.5M has been approved by the grant-making foundation, and is expected to arrive by March 23rd 2019.
4. The first $1 Million was matched in 2018 (inclusive of some of the pledges), and now all pledges need to be fulfilled to reach the additional $1.5 Million needed to meet the $2.5 Million goal.
5. Of the $1.5 Million amount for 2019, NDF still needs to raise $800K (and collect unfulfilled pledges from 2018)
6. Therefore, NDF must plan another fundraising event for Fall 2019, starting now.
7. If you are interested in attending, sponsoring or volunteering at one of our popular events, please email events@CureHIBM.org

What's going on with the SCIENCE? (ManNAc & Gene Therapy)

1. The proposals and requests for funding were vetted by the NDF Scientific Review Committee, and approved by the NDF Board of Directors. See press release linked here.
2. All grantees have been contacted and have begun their respective projects.
3. $150K was gifted to the NIH to expedite the final phase of ManNAc. This treatment will help the greatest number of patients by **slowing down the progression** of GNEM.
4. All other scientific **projects are directly linked to GENE THERAPY,** and as listed in the most recent press release, add up to approximately $550,000 (not including the gift fund for NIH).

When are you going to start GENE THERAPY?

1. Gene therapy is already in progress and has been since 2017.
2. Gene therapy is a multi-step process:
   Pre-clinical work including research and development and pre-clinical tests; followed by filing an IND and Phase 1, Phase 2, Phase 3 →Market.
3. NDF is now in the second step of the pre-clinical stage, with work being carried out on both the research and development and pre-clinical testing phases simultaneously.
   These have to be completed before the FDA can approve or "greenlight" the clinical stage (IND).
   A Pre-IND document (a request to the FDA of what additional pre-clinical work must be completed before filing an IND) was filed by Dr. Mendell via Nationwide (NCH) and was paid for by NDF.

4. In spite of what may have been told to patients by certain physicians/scientists, the FDA still had a number of questions and comments in response to the Pre-IND filing which need to be answered before an IND can be filed.
5. NDF has been working to answer the comments and questions from the FDA and is NOW in a position to fund scientists to provide further answers to the FDA's questions. The work on development of a new mouse model, the work on biomarkers and whole genomic sequencing will maximize our chances of getting to IND and Phase 1.
6. Once these studies are completed (or at least have results to share) we will apply for the IND.

Who else are you working with?

1. Everybody. NDF's main goal has been to collaborate with ALL known academic institutions, industry partners and individual professionals knowledgeable about GNEM, worldwide. As the world's leading organization for funding science related to GNEM, our goal is to centralize all data in order for others to build upon. To date, we have succeeded in pooling all global resources and as of Q1, 2019, we have representation in 26 countries and counting. . .
2. These organizations include (but are not limited to) UC Regents, University of California, Yale University, Nationwide Children's Hospital, ARM, The National Institutes of Health (NIH), MDA, Ohio State University, Hadassah Medical Center (Israel), Newcastle University (UK), MDUK, University of Ottowa (Canada), National Center for Neurology (NMD Japan), World Without GNE (India), Istanbul University (Turkey), and Pusan University (S. Korea) to name a few.

What are you doing with Bio-techs?

1. Academic Institutions (like NCH) or Principal Investigators (PIs), are an integral part of the process, however as academic institutions they can only take us, at best, to Phase 1.
2. In order to make Gene Therapy accessible to all patients, we have to get through each phase with the help of PIs, approval from the FDA, and financial and scientific support from BIOTECHS interested in investing in GNE Myopathy.
3. It costs at least $5M to get to Phase 1.
4. It costs at least $120M to get from Phase 1 to Market.
5. Neither NDF, nor any one doctor or PI is in a position to go to market without Biotech buy-in.
6. NDF is now, and has been, in discussions with best-of-breed Biotech partner prospects, specializing in rare muscle gene therapy.
7. **In short:** NDF's goals for 2019 & 2020 are to: a) answer the FDA's questions about gene therapy for patient testing b) apply for an IND and c) lock down an agreement with a biotech partner.

What are other NDF PROGRAMS and why do you spend money on them?

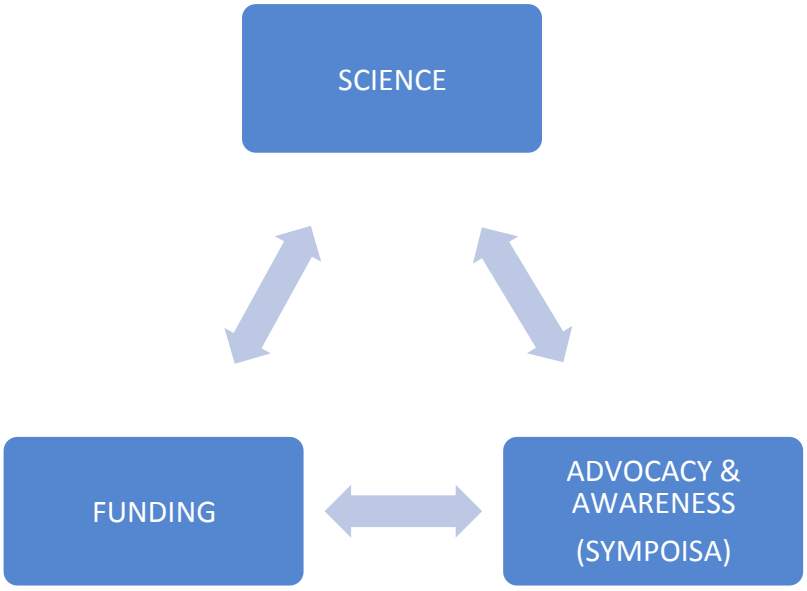
1. NDF's commitment is to its patient population and, as such, cannot sit idly by to wait for a cure.
2. By bringing patients, caregivers, doctors and scientists together, we solve multiple problems while doing the right thing:

A. Education between scientists about the disease is made possible at NDF's symposia, and is largely credited for creating collaboration among scientists, many of whom refused to work together just a few years ago. **This expedites scientific progress.**

B. Awareness of the disease leads to proper and timely diagnosis which, in turn, helps expedite interest from doctors, scientists, and ultimately INVESTORS and Biopharma, without which a cure is untenable. This increases the number of identifiable patients, which increases the money that can be made by industry and **expedites treatments and a cure. Having more identified patients and getting information on their disease state and progression will help NDF to generate a natural history study that is highly desired by the biotechs and the FDA.**

C. Advocacy for patients and their families is not only the right thing to do, but also promotes NDF as a solid, compliant and effective nonprofit organization according to watchdog groups, and donors alike. It is directly because of our work on these programs that NDF staff were able to apply for and win grants, such as the recent one for $2.5 Million.

Everything is connected!



Thanks for your continued support as we work to solve problems for all GNEM patients and stakeholders.