Sponsoring Research Leading to Development of Cure and Better Assistive Devices

There is no therapy/treatment available for more than 99% of rare genetic disorders. We believe that lack of awareness among scientists, dedicated funding and research resources have contributed to this dismal picture. Our main goal is to help develop a cure for GNE Myopathy and other rare genetic disorders. Apart from disease specific therapies, we also plan to develop platform technologies that can be a basis for finding cure for large number of genetic disorders. We plan to sponsor targeted research by scientists who are in the forefront in their respective areas. All research related activities will be carried out in consultation with distinguished members of our Scientific Advisory Committee. The composition of the committee is given in our website.

Some of the specific areas of research we are looking into are:

- Development of a muscle specific gene therapy vector with minimum toxicity and side effects.
- Development of stem cell lines carrying mutations from patients and use of these for understanding disease biology and drug screening.
- Development of technologies and facilities for large scale production of Good Manufacturing Practice (GMP) quality enzymes, vectors and mRNAs for clinical trials.
- Repository of animal models that mimic disease biology.
- Bank of tissues and fibroblast cell lines from patients.
- Repository of small molecule library for drug screening.
- Setting up of a clinical research unit specialized in clinical trials of different therapies.
- Database of research resources in the area of rare genetic disorders.

Your contributions can go a long way towards funding a cure and transforming the lives of many rare disease patients.