



NUBPL: A Mitochondrial Disease caused by mutations in the NUBPL Gene: One \$120,465 grant is available for research related to treatments or cures of this form of mitochondrial disease, and/or the creation of natural history studies of the disease to advance future clinical trials or research studies. This grant can advance research or projects already in progress or be used to initiate new research or studies. Examples of priority topic areas include developing, advancing, or continuing disease models, identifying potential therapeutics whether they consist of drugs, vitamins, diets, or supplements that are currently in the market or the development of novel molecules, studying the effectiveness of therapies currently in use for mitochondrial disease in this form of the disease (including components of what is known as the "Mitochondrial Cocktail"), studying or establishing gene therapies, establishing outcome measures to be used in clinical trials, and developing other essential resources to substantially prepare the NUBPL community for clinical trials. This grant is made possible by the NUBPL Foundation, Inc.

