Gene Editing/Cell Therapy Initiative ($35,000)
The New York Stem Cell Foundation (in partnership with Baylor College of Medicine)

The INADcure Foundation recently expanded its partnership with NYSCF to explore the possibility of developing a cellular therapy for INAD patients using gene-corrected neurons. In collaboration with CRISPR pioneer Dr. Feng Zhang of MIT, NYSCF has successfully gene-edited a patient iPSC line to correct their PLA2G6 mutation. The next step is differentiating this line into neurons, to verify that the gene edit successfully reverses disease phenotypes. If a proof of concept is established, it will open doors to pursue a cell replacement therapy.

INAD BioBank ($50,000)
The New York Stem Cell Foundation

The INADcure Foundation has created an INAD biobank in partnership with the New York Stem Cell Foundation Research Institute. A biobank is a type of biorepository that stores patient-specific biological samples for use in research. Establishing this biobank allows for research ready patient and unaffected family member cells to be easily accessible and delivered to scientists.

Compound Screening ($75,000)
Dr. Hugo Bellen/Bellen Lab at Baylor College of Medicine

Dr. Hugo Bellen is an Investigator of the Howard Hughes Medical Institute and Distinguished Service Professor at Baylor College of Medicine (BCM) in the Departments of Molecular and Human Genetics and Neuroscience. Dr. Bellen's current research focuses on elucidating pathogenic mechanisms of neurodevelopmental and neurodegenerative diseases. The INADcure Foundation is currently funding his lab to screen compounds on fruit flies and on cells of affected children in hopes of identifying potential treatments to stop or slow the progression of the disease.

PLAN Ready Natural History Study ($150,000 / $30,000yr)
NBIA Research Group at Oregon Health and Science University
Project status: **<u>Currently funding 2nd year of a 5-year study</u>**

The purpose of this study is to help researchers better understand the natural history of PLAN/INAD, meaning how symptoms appear and change over time. By studying individuals with INAD the NBIA Research Group at OHSU hopes to identify disease markers that can be used in future clinical trials. A disease marker is any symptom or measurement that happens reliably in a disease, changes predictably with disease progression, and becomes "better" with successful treatment. Natural history studies provide data that serve as the foundation for future drug trial, are required by the FDA, and can hold up a study from starting if natural history data is not sufficient. Supporting this work is essential in order to ensure the rapid advancement through the FDA's clinical trial application process when potential new drugs or treatments (like gene therapy) are ready for clinical trials.

Bisous For Léo and the INADcure Foundation are committed to bringing breakthrough treatments, and one day a cure, to all children living

with INAD. To help, please visit: www.BisousforLeo.org