

## **Entering the Therapeutics Era for NBIA Disorders**

The era of Mendelian disease gene discovery has largely ended. For the NBIA disorders, more than 95% of patients can be diagnosed with a specific genetic etiology. Genetic delineation of the NBIA disorders has enabled the development of natural history datasets, animal disease models, biological insights into etiologies, and robust family advocacy communities. Still, treatment for these disorders is strictly symptomatic and largely unchanged from decades ago. That is about to change. We are on the cusp of interventions that hold enormous potential for a disease-modifying outcome. Therapeutics programs in PKAN and PLAN that are based on robust studies of animal disease models are the most advanced, and experience with these will inform work to develop treatments for all NBIA disorders. We have moved into the era of therapeutics for the NBIA disorders.