
Research Focus Areas:

Beta-propeller protein-associated neurodegeneration (BPAN)/Neurodegeneration with Brain Iron Accumulation Disorder (NBIA) disorders: Two pilot grants for \$60,000 each are available for clinical and translational research studies related to the detection, diagnosis, or treatment of this rare, X-linked disorder caused by mutations in WDR45. BPAN typically is recognized in early childhood with delayed development and seizures. In adulthood, people with BPAN develop rapidly progressive parkinsonism. At the present time, symptoms may be treated but there is no cure.

Grants are expected to generate essential information for the scientific community to advance knowledge about BPAN disease processes, and to produce preliminary data to enable national and international funding to carry the work forward. Examples of priority topic areas include: developing and exploiting disease models including computer models, identifying biomarkers, delineating the molecular cascade that leads to early cellular changes, developing rational therapeutics, establishing outcome measures to be used in clinical trials, and developing other essential resources to substantially prepare the BPAN community for clinical trials. Natural history studies proposals should reflect knowledge of existing, ongoing studies and include a statement indicating how the proposed study would complement or integrate with existing studies. Moreover, proposals for natural history studies must have a component that includes participation in the TIRCON International NBIA Patient Registry & Biobank. This grant is made possible by Team NBIA Disorders and BPAN families with the NBIA Disorders Association.