Research Focus Areas:

SETBP1: The purpose of this RFA is to promote understanding of underlying disease mechanisms and pre-clinical development of potential therapies and tools for SETBP1 haploinsufficiency disorder (SETBP1-HD). **One $88,740 grant available.** Areas of interest include, but are not limited to:

- Identifying molecular pathways involved in this disease
- Investigating repurposing of existing FDA approved drugs as a treatment for SETBP1-HD
- Identifying novel drugs or therapies for SETBP1-HD
- Investigating language, cognitive, behavioral and/or attention clinical profiles through natural history studies to further delineate the SETBP1-HD phenotype and develop diagnostic and/or predictive biomarkers for clinical trials with a preference for virtual administration with multi-language support

In addition, applicants are encouraged to collaborate with existing SETBP1 researchers and to leverage existing disease models (e.g. animal models at JAX, patient-derived cell models at SFARI or COMBINEDBrain bioreposities, etc.) to assess therapeutic impact. This grant is made possible by Team SETBP1Strong and SETBP1 Society.