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 grant for $91,664 or two grants for $45,832 are 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
- Identify Proteomics, Metabolomics, & Transcriptomics biomarkers to be used in future clinical trials. A biomarker is broadly defined as any objective modality that can measure disease activity and could include quantified biological variables (e.g., blood- or urine-based tests), novel imaging techniques, or patient-reported outcomes.

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 biorepository, etc.) to assess therapeutic impact. This grant is made possible by Team SETBP1Strong and SETBP1 Society.

*Please submit a proposal for the total amount of $91,664. The ODC may choose to fund two awards at $45,832 each, at which point we will request a revised work plan and budget.