

**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, also known as SETBP1 disorder. One \$91,466 grant or two \$45,733 grants 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 disorder
- Identifying novel drugs or therapies for SETBP1 disorder
- Investigating language, cognitive, and attention clinical profiles through natural history studies to further delineate the SETBP1 disorder 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 clinical trials

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 \$91,466. The ODC may choose to fund two awards at \$45,733 each, at which point we will request a revised work plan and budget.