

Targeted Call for new proposals specifically focused on CRB1

In addition to our broad call for applications that align with our research priorities, the Foundation is pleased to call for new Program Project Award (PPA) proposals that specifically address gaps in our understanding of CRB1.

Through landscape analysis and discussion with key stakeholders, we have identified the following gaps in CRB1-related research. Applications that propose projects that address these gaps are especially welcome.

Gap 1: Incomplete understanding of fundamental CRB1 biology.

Possible questions include:

- When/where are the different CRB1 isoforms (RNA and protein) expressed in human cells and/or other relevant animal models?
- What are the differential roles of the isoforms?
- How do mutations in and/or CRB1 dysfunction lead to degeneration?

Gap 2: Lack of faithful models to understand CRB1 biology and disease and for testing therapeutic agents.

Possible approaches include:

- Creation and/or phenotyping of novel animal models, with a preference for large animal models
- Creation of a CRB1 patient biorepository for the generation and characterization of iPSCs and retinal organoids

Gap 3: Incomplete understanding of factors contributing to patient variability and lack of clinical tests that can serve as endpoints in interventional clinical trials.

Possible outstanding questions to be addressed include:

- What contributes to the variability in patient presentation and progression?

- Given the variability in patients, what is the most robust endpoint for an interventional clinical trial?

The PPA is designed to support collaborative, multi-disciplinary, research studies that engage investigators with different expertise and resources. The PPA is intended to support studies that are too large or complex for a single investigator to undertake in a reasonable amount of time and to address gaps in our current knowledge or therapeutic options. The PPA must be unified around a single, well-articulated hypothesis and a clearly defined deliverable(s), relevant to the mission of FFB. The teams supported by a PPA must be tightly integrated and sharply focused, freely share data and creative ideas, and each be essential to the common goal. Applications focused on CRB1-related research will follow the same timeline and be reviewed by the same panel as our standing PPA competition.