

### <u>Individual Investigator Research Awards – Targeted Call for New</u> Proposals

The Foundation Fighting Blindness (FFB) expects to fund a limited number Individual Investigator Research Grants to be awarded June 2023.

If you are interested in being considered for an award, submit a Letter of Intent (LOI) and short, no longer than five pages, Curriculum Vitae (NIH Biosketch is acceptable) to FFB by 10/27/2022 via the FFB application portal [https://www.onlineapplicationportal.com/blindness].

The following sections must be entered into the designated application portal fields:

- 1. Title of Project
- 2. FFB Research Priority Area (RPA) (ONE ONLY, see below)
- 3. Inherited Retinal Degenerative Disease(s) (or dry AMD) that this research impacts and why this research is important to and will make a significant difference in achieving the Foundation's mission.
- 4. Overall research, goals and hypothesis to include the Specific Aims and rationale proposed for FFB Grant funding (it is recommended that the specific aims are listed and rationale stated)
- 5. Stage of Development
- 6. Overall Description of Application or research proposal
  - a. No more than 3 figures/tables are permitted but not required. Figures/tables can be uploaded on the Figures Upload page. doc or pdf acceptable
- 7. Reference list
- 8. CV for the Principal Investigator only. 5 page NIH biosketch format is acceptable. Curriculum Vitae is not included in character limits.

A budget is not required for the Letter of Intent



Use the Print Application tab to preview your application in pdf format. Your signature will be added to the pdf when the Submit Application page is completed.

Email confirmation of submitted application will be sent immediately from <a href="mailto:blindness@onlineapplicationportal.com">blindness@onlineapplicationportal.com</a>. Add this address to your safe sender list to avoid emails being sent to your SPAM folder.

#### **Description:**

Individual Investigator Research Awards are designed to concentrate research in areas that will have the greatest potential to move towards treatments and cures for the **inherited orphan retinal degenerative diseases** and **dry age-related macular degeneration (dAMD).** N.B.: FFB does not support research for neovascular AMD or diabetic retinopathy. The Foundation has identified Research Priority Areas (RPA) that align with its mission and this targeted open call for application is to address specific gaps identified in current retinal disease research. While applications addressing the areas of particular interest below will be given priority consideration, the FFB will also consider proposals for highly novel research that do not fit easily within these goals. The LOI for such proposals must clearly explain why the research is likely to lead to prevention, treatments or cures for the orphan inherited retinal degenerative diseases.

Individual research awards are available in **the following Research Priority Areas:** 

## **RESEARCH PRIORITY AREAS (RPA)**

## **Novel Medical Therapies (NMT)**

Develop drug therapies that retain retinal function and structure in retinal degenerative diseases. This includes the creation and development of improved animal models of human disease, better



functional testing of drug effectiveness, and novel drug delivery systems.

<u>Applications that target the following areas are of particular interest:</u>

- Develop pan-disease therapeutics
- Develop high throughput phenotypic drug screening tools (markers, target, etc) relevant to the human orphan inherited retinal degenerative diseases.

#### Gene Therapy (GT)

Develop and optimize viral and/or non-viral gene delivery systems for use in the treatment of dominant, recessive and X-linked retinal degenerative diseases. Demonstrate efficacy and safety using pre-clinical models in preparation for human clinical trials. FFB limits its funding to the development of the technology that will benefit gene-therapy strategies.

Applications that target the following areas are of particular interest:

- Develop methods of gene delivery that can:
  - Target specific retinal cells
  - Efficiently transduce all cells of a given type in the retina
  - Deliver large gene constructs that may contain large coding regions and/or large gene control elements
- Develop clinically relevant approaches for genome editing

## **Cell and Molecular Mechanisms of Retinal Disease (CMM)**

Basic research that improves our understanding of the nature and cause of disease in inherited retinal degenerations so that improved therapies for the prevention of vision loss can be developed.



# <u>Applications that target the following areas are of particular interest:</u>

- Delineate pathways that link mutations in multiple different genes to common disease mechanisms, with the goal of identifying pan-disease therapeutic targets.
- Develop and characterize cone-rich and/or non-rodent animal models for the RDD that are relevant to human RDD.

#### Genetics (GE)

Identify disease-causing mutations in inherited retinal disorders, in part by integrating comprehensive genetic testing into routine clinical care. Identify inherited risk factors for age-related macular degeneration (AMD) and the relative contributions of associated genetic and non-genetic factors (e.g. lifestyle), sufficient to incorporate into treatment and preventions.

## Applications that target the following areas are of particular interest:

- Develop and validate faster, more accurate and less expensive methods to identify mutations in both known and unknown genes implicated in the orphan inherited retinal degenerative diseases
- Develop clinically relevant approaches for genome editing

## Clinical-Structure and Function (CL)

Clinical research that develops improved technology and standardizes processes to establish relationships between clinical retina function and retina structure in retinal degenerative diseases and enables early disease detection.

N.B.: If a clinical application focuses on a therapeutic intervention, the application should identify and submit their application using the most relevant RPA for that therapy, such as GT, or NMT, instead of using CL.



# <u>Applications that target the following areas are of particular</u> interest:

- Develop and validate diagnostic technology and endpoints for clinical trials, that include, but are not limited to:
  - Natural history studies that correlate genotype and phenotype
  - Biomarker identification
  - Improvements in retinal imaging

### Regenerative Medicine (RM)

Develop strategies that provide functional rescue or replacement of degenerating or dead retinal cells that can lead to the slowing and prevention of vision loss, or the restoration of lost vision.

Applications that target the following areas are of particular interest:

 In vivo transdifferentiation studies, or lineage reprogramming, in which retinal cells are transformed into another retinal cell type without undergoing an intermediate pluripotent state or progenitor cell type.

#### **Eligibility:**

Applicants must hold a Ph.D., M.D., D.M.D., D.V.M., D.O., or equivalent degree and have a faculty position or equivalent at a domestic or foreign: non-profit organization, or public or private institution, such as a university, college, medical school, hospital, research institute, or laboratory. Individuals from underrepresented racial, ethnic and gender groups, as well as individuals with disabilities, are always encouraged to apply.

#### Award:

The award will be approximately \$100,000 per year up to three years. The award may be used to support the salaries of research trainees (graduate students, postdoctoral or clinical fellows), technical staff and research supplies. Partial support for the Principal Investigator's salary is permitted but is not to exceed 20% of the total annual award. The Foundation Fighting Blindness does not provide funds for equipment or indirect administrative costs. A budget is not required for the Letter of Intent.



**Letter of Intent Submission** 

Applicants must submit the completed LOI electronically by 10/27/2022 11:59PM EST via the FFB Application Portal. Access the FFB Application Portal through the FFB website,

https://www.fightingblindness.org/individual-investigator-research-award.

Automated acknowledgement of receipt of LOI will be sent upon submission.

The Letters of Intent will be reviewed for scientific quality and relevance to FFB's mission and current research priorities. Letters addressing the targeted area identified above and showing clear relevance to translational studies that can accelerate the path toward clinical trials will receive priority consideration. If your Letter of Intent is selected, the FFB will contact you to request a full application by January 2023. Full applications will be due on 2/27/2023.

PLEASE USE YOUR OWN E-MAIL ADDRESS IF POSSIBLE WHEN SUBMITTING THE LETTER OF INTENT SO THAT WE CAN INFORM YOU IN A TIMELY FASHION IF YOUR APPLICATION HAS BEEN SELECTED FOR SUBMISSION OF A FULL PROPOSAL. IN ADDITION, PLEASE ADVISE US AS SOON AS POSSIBLE OF CHANGES IN E-MAIL ADDRESSES.