FOUNDATION FIGHTING BLINDNESS
Together, we're winning.

Individual Investigator Research Awards and Clinical Innovation Awards– Targeted Call for New Proposals

The Foundation Fighting Blindness (FFB) expects to fund a limited number Individual Investigator Research and Clinical Innovation Awards to be awarded June 2024.

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 10/26/2023 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 blindness@onlineapplicationportal.com. 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.

The Foundation Fighting Blindness will award up to four **Clinical Innovation Awards** to advance options for endpoints in IRD clinical trials that might be accepted by regulatory agencies for improving IRD patients' conditions or slowing disease progression. Priority consideration will be given to those proposals that plan to:
(1) establish sensitive and reliable outcome measures or biomarkers to demonstrate change over a time period spanning no more than 2 to 3 years;
(2) develop and apply new technology to measure retinal structure or function in inherited retinal degenerations where changes over time are greater than measured variability;

(3) incorporate patient reported outcomes or patient preferences;
(4) establish relationships between measures of retinal function and structure with the goal of understanding the relationship between genotype and clinical phenotype;
(5) establish a framework for grading/assessing the severity of multiple IRDs that can be used in clinical trials for assessing progression of disease;
(6) leverage data collected through the FFB Consortium;
(7) improve retinal imaging or grading of images (increased reliability, sensitivity, efficiency, etc)
(8) develop performance-based tests that are suitable for multicenter studies
(9) develop endpoints appropriate for early stage (e.g., pediatric patients) or late stage disease (e.g., relevant for cell therapy or optogenetics)

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

(**NOTE**: studies focused on wet AMD and diabetic retinopathy are **not** eligible for support by the Foundation Fighting Blindness).

Individual Research Awards are available in the following Research Priority Areas:

Research Priority Areas

1. **Novel Medical Therapies (NMT)**
    a. The goal is to promote the development of new therapies (small molecules) that slow or prevent the loss of retinal function
    b. Applications that target the following areas are of interest:
        i. Development or testing or therapeutics for photoreceptors or RPE-centered retinal degenerations.

ii. Development or testing of therapeutics with broad spectrum or mutual-independent applications.

iii. High-throughput phenotypic drug screening tools (markers, target, etc.) relevant to the human orphan inherited retinal degenerative diseases.

iv. The development of improved animal models of human disease to enhance functional testing of drug effectiveness and novel drug delivery systems.

2. **Gene Therapy (GT)**

   a. The goal is to find a viral and/or non-viral gene delivery system(s) to treat dominant, recessive, and X-linked retinal degenerative diseases. The application must also address a plan to evaluate efficacy and safety using pre-clinical models in preparation for human clinical trials. The Foundation limits its funding to the development of the technology that will benefit overall retinal gene-therapy strategies.

   b. Applications that target the following areas are of interest:

      i. Gene therapy delivery methods

         i. Target specific retinal cells

         ii. Effectively and efficiently transduce all relevant retinal cell types

         iii. Develop strategies to deliver complex constructs (large DNA, gene editing tools in the form of DNA, mRNA, or protein)

      ii. Clinically relevant approaches for gene editing

3. **Cell and Molecular Mechanisms of Retinal Disease (CMM)**

   a. The goal is to expand the basic understanding of inherited retinal degenerations and dry age-related macular disease etiology toward mitigating or curing vision loss.

b. Applications that target the following areas are of interest:

   i. Pathways linking mutations in multiple genes to common disease mechanisms, with the goal of identifying pan-disease therapeutic targets

   ii. Develop and characterize cone-rich and/or non-rodent animal retinal degeneration disease models relevant to human retinal degeneration diseases

   iii. Pathway identification and their interactions with other pathways to cause AMD lesions and AMD pathobiology.

4. **Genetics (GE)**

   a. The Foundation Fighting Blindness supports research to identify a.) genes and mutations causing inherited retinal diseases (IRDs) and b.) genetic factors contributing to atrophic (dry) age-related macular degeneration (AMD). For both, the goal is to incorporate these finding into clinical care, and to foster development and application of treatments and cures. Improved clinical care, and treatments and cures, must be potential outcomes of all supported research.

   b. Applications that target the following areas are of interest:

      i. Develop and apply advanced genetic testing methods to find new IRD genes, novel IRD mutations, and IRD mutations refractory to conventional genetic testing such as structural rearrangements and regulatory variants.

      ii. Develop laboratory and/or computational tools to identify and confirm or exclude pathogenic mutations, such as variants of uncertain significance.

      iii. Identify genetic and environmental factors modifying clinical expression of IRD mutations.

      iv. Continue to identify and understand genetic risk factors associated with dry AMD, including both rare and

polymorphic variants; and further define risk alleles, risk haplotypes and related genetic factors.

5. **Clinical-Structure and Function (CL) (***Select Clinical Research Area for the Clinical Innovation Award)**
    a. The goal is to advance research that develops improved technology and standardizes processes to establish relationships between retinal function and 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.
    b. Applications that target the following areas are of particular interest:
        i. Develop and validate diagnostic technology and endpoints for clinical trials, that include, but are not limited to:
            i. Multicentered natural history studies that correlate genotype and phenotype
            ii. Biomarker identification
            iii. Improvements in retinal imaging or grading of images
        ii. Proposals that leverage data collected through the FFB Consortium

6. **Regenerative Medicine (RM)**

    The goal is to develop strategies to rescue or replace degenerating or dead retinal cells leading towards restoration of lost vision, or slowing and/or prevention of vision loss.

Eligibility:
Applicants must hold a Ph.D., M.D., D.M.D., D.V.M., D.O., O.D., 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 10/26/2023 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 2024. Full applications will be due on 3/6/2024.

PLEASE USE YOUR OWN E-MAIL ADDRESS IF POSSIBLE WHEN SUBMITTING THE LETTER OF INTENT SO THAT WE CAN INFORM

FOUNDATION FIGHTING BLINDNESS
Together, we're winning.

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.