

# Retina UK Research Strategy

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Retina UK is a Charitable Incorporated Organisation

Registered charity number: 1153851

## Our Research Mission

To stimulate and support high quality medical research with the aim of increasing scientific understanding of retinal dystrophies, including RP and associated syndromes, and finding treatments or cures for those conditions which, when developed, are accessible to anyone affected.

## Our Vision

We aspire to a world in which people with retinal dystrophies will have access to treatments or a cure and will not lose their sight.

## Our Values

The charity is committed to focus on its objectives, transparency in all matters, and collaboration with other organizations, fair business practices and equality of opportunity.

## Background

Retina UK has been funding medical research since the late 1970s. Retina UK was founded in 1975 by a number of people with retinitis pigmentosa (RP). The group was concerned at the lack of knowledge about RP in the medical profession, the lack of a treatment or cure.

Retina UK has since evolved into a respected medical research charity and a nationwide organization providing support and information. We fund cutting edge research into the causes of and potential treatments for the disease. The charity is currently working toward a strategy that will significantly increase the value of the investment possible. The charity will also be undertaking capital fundraising campaigns to support the advancement of retinal dystrophy research.

## Research aims and objectives

Retina UK welcomes high quality research applications in any area of research related to retinal dystrophies but priority will be given to projects with promise for progressing the development of treatment strategies or slowing down the progress of sight loss. This includes basic laboratory research investigating disease mechanisms, preclinical studies in animal and cell models and genotyping and phenotypic investigation of patient populations as a prelude to clinical trials.

Retina UK is focussed on understanding the causes of retinal dystrophies and identifying cures, treatments and preventions; allowing patients to either maintain or restore vision through high quality relevant research. The specific aims are:

- To support and stimulate high quality research into the cause, treatment, prevention and cure of inherited retinal dystrophies.
- To support the objectives outlined in the Sight Loss and Vision Priority Setting Partnership (Appendix III).
- To follow AMRC guidelines and retain our NIHR Partner status.
- To stimulate research in identified areas of importance.
- To support the early detection of RP and retinal dystrophies.
- To encourage and stimulate the dissemination of research results and relevant collaborations to avoid duplication of work and drive advancement of treatments, diagnosis tools and prevention aids.
- To maintain a robust and accurate assessment process, including relevant scientific recommendation and peer review analysis.

To realise this aims Retina UK will:

- Continue to fund innovative breakthrough research.
- Continue to focus on research specifically interested with the diagnosis, treatment and prevention of retinal dystrophies.
- Continue to strive for standard classification of retinal dystrophies diagnosis and register those patients.
- Continue to have a robust, peer reviewed application process – ensuring the highest quality research.
- Continue to strive to raise more income to fund more vital research and effectively communicate this to potential research investigators.
- Fundraise and facilitate fundraising against capital projects and specific research activities. This includes facilitation of major donations for specific research questions or researchers.
- Facilitate effective communication between patients, scientists and clinicians to promote mutual understanding and potential participation in studies, where appropriate.
- Develop collaborations with industry and other patient and public sector organisations to prepare for effective delivery of future treatments to patients.

## Appendix I: Criteria and Types of Funding

### **Fundamental Criteria**

- High quality research project involving respected and experienced research leaders.
- Relevant to Retina UK research objectives and aims as set out previously in this document.
- Value for money.
- Advances the objectives of Retina UK. (This may include projects that are helpful for fundraising purposes).
- The research must be legal, and subsequent resulting treatment recommendations also legalised.
- The application must be made by a professional regulatory or statutory body (or equivalent) accredited program.
- The investigator and their institution must accept and adhere to Retina UK's Research Grant Terms & Conditions.

### **Other Criteria**

- Funds are available.
- Applicants should have a good track record of obtaining funding from other sources and be recognised amongst their peers for their research. Retina UK may support promising early career researchers.
- Where applicants have received previous grants from Retina UK there must have been good outcomes and publications related to the funded project.
- Priority will be for project grants and studentships. Programme grants, fellowships or grants for equipment may be approved if finance allows.

- Retina UK favours projects that attempt to stimulate and trigger new areas of work and will consider pump-priming grants for new areas of research.
- From time to time the charity may call for applications in a specific area of research. This includes calls to commission appropriate research on behalf of a donor who will fully fund the project. Such projects must be in keeping with Retina UK's aims and objectives. (See Appendix III)
- There are no geographic location restrictions on applications.

### **Types of funding**

- Program (>£200K) 3 - 5 years
- Project (<£200K) < 3 years
- PhD / Post-Doctoral investigations (>£100K) varying length
- Small Project (Innovation) (<£100K) varying length

Retina UK is also funding pioneering research carried out at specific centres of excellence in the UK with an AMRC compliant process in place to ensure that the research meets high standards of scientific merit.

### **Grant Process and Administration**

The MAB operates according to the Protocol below:

- Funding applications can be submitted at one of three specified dates per year: 1<sup>st</sup> September, 1<sup>st</sup> February, 1<sup>st</sup> June. The 1<sup>st</sup> September round will be publicised, with the other two providing capacity for responsive and timely funding where necessary; Retina UK reserves to right to publicise other rounds as necessary to ensure equitability. Preliminary enquiries from researchers are required in the form of a brief letter of intent. Such letters are assessed by

the MAB and invitations are issued as appropriate to submit full applications and budgets.

- Full applications for grants are sent to the Chairman of the MAB and all applications are subject to peer review.
- The MAB discusses the applications and makes recommendations to the Board of Trustees where the final decision is made. Successful applicants are informed and further grant administration is in the hands of the office in Buckingham, which refers to the MAB on scientific matters. All grant holders are required to submit an annual report to the charity and a final report on completion of the project.
- Grants are awarded for the full length of the project on the proviso that a satisfactory annual report is submitted to Retina UK.

#### Summary of elements funded:

- Salaries
- Consumables
- Project-related expenses only
- Animal subjects and related costs
- Space rental

#### Elements not included:

- Capital purchase (permanent laboratory equipment or materials). Exceptions may be made in certain cases where restricted funding is involved.
- Non-project related expenses
- Utility costs
- Annual, maternity or sick leave payments
- Publishing costs
- Parking or travel allowances at general place of work
- Administrative support

After the project is approved:

- The funding will be made available from the approval date and be payable upon invoice from the institution.
- Six monthly reports will be expected by the Board of Trustees, and satisfactory completion and submission is needed to guarantee continuation of funding.
- The Retina UK Communications and Fundraising teams will require regular updates to further stimulate income and raise profile.
- Post-research review – evaluate the effectiveness of our funding, advancing further research and provide feedback on our grant application process.

### **Restricted funding**

Retina UK recognises that members of the inherited sight loss community or biotechnology industry may wish to provide funding, via Retina UK, to answer a specific research question or to support the work of a particular researcher.

In order to maintain high standards of scientific integrity in relation to the research we fund and facilitate, applications for projects supported by restricted donations will be subjected to Retina UK's usual stringent peer review process.

Full details of our processes for managing restricted funding are available in our Policy for Restricted Research Funding – see Appendix III.

Under no circumstances will Retina UK accept restricted funds for a project prior to it being approved.

## Appendix II: Medical Advisory Board Protocol

### **Remit of the MAB**

- To assess preliminary grant applications and invite full submissions.
- To assess full grant applications which have been peer reviewed and make recommendations to the Board of Trustees as to funding.
- To operate to the grant application and approval timetable, as outlined in the MAB Protocols document.
- To meet face to face on an annual basis and conduct virtual meetings as necessary to review applications at other times of the year and to minute those meetings.
- To assess annual progress reports from funded projects and make recommendations to the Board of Trustees as to continuation funding.
- To provide advice to the Board of Trustees concerning any research governance issues relating to research funded by the charity.
- To review documentation produced by Retina UK relating to clinical and scientific aspects of retinal disease.
- To regularly review this policy / protocol document and the medical research section of the charity's strategic plan and make recommendations to the Board of Trustees concerning research priorities.
- To assist and support the Retina UK office in matters related to grant administration where a scientific input is required.
- To assist and support Retina UK fundraisers with scientific inputs to donor applications.
- To provide Retina UK with medical statements, in layman's language, on current research issues and hot topics.

- To recommend to the Board of Trustees the selection of our representatives on the Scientific and Medical Advisory Board (SMAB) of Retina International.
- To ensure the Executive Team is kept informed as to major research developments for onward transmission to members as appropriate.
- To assist the administrative team in dealing with any enquiry that needs a medical or scientific response.

### **Membership of MAB**

All MAB members are appointed by the Board of Trustees, based on a recommendation from the MAB Chairman. The membership of the MAB will include:

- A Chair (a scientist or clinician).
- A Deputy Chair (a scientist or clinician).
- A minimum of three and a maximum of five other members who should be leading clinicians and / or scientists involved in research in genetics, ophthalmology, cell biology or a closely related discipline.
- Co-opted members as appropriate if additional specialist expertise is required.

The Chairperson and Deputy Chairperson may not apply for Retina UK grants during their period of tenure. Other MAB members may be Retina UK grant holders but will excuse themselves from the MAB's discussions of their grants and projects and leave the room. All members of the MAB are expected to declare any other conflict of interest, as described in the MAB Protocol document.

All MAB members including the Chairperson and Deputy Chairperson are appointed for a period of three years renewable for further periods of three years.

## **Applications and Meetings**

The MAB will meet (virtually or in person) after each submission deadline to consider grant applications and to review progress reports from existing grant holders. The MAB will also consider any issues raised by the Board of Trustees or the Executive Team.

Potential applicants will submit a short (one page) outline of the intended research project to the Chairperson of the MAB, prior to making a full application by invitation.

Full grant applications will be peer reviewed (see below) by three independent referees chosen by the Chair of the MAB with advice from other members as necessary. Applications, peer reviews and annual progress reports will be circulated to MAB members at least 14 days in advance of the MAB meeting.

The Chief Executive or another member of the Executive Team will be invited to attend.

The MAB meeting will be held at an appropriate time so that recommendations can be considered by the Board of Trustees at their meetings. The MAB will make formal recommendations in writing as to funding. This document will be submitted to the Board of Trustees in the form of a meeting paper or papers, or minutes of the MAB meeting, 14 days prior to the Trustees' meeting.

Apart from the meetings, further communication between MAB members is encouraged by telephone, teleconference and email as needs arise, for example, to address issues arising or to determine a response to significant research news.

From time to time the medical trustees may convene special meetings of invited clinicians and scientists to discuss key

issues related to RP research and the development of cures and treatments. Recommendations that emerge for changes to research policy and priorities will be made to the Retina UK Board of Trustees. The Chief Executive or another member of the Executive Team will be invited to attend such meetings.

### **Peer Review Process**

All Retina UK grant applications are subject to peer review. Referees may be selected from relevant experts in the field of the application under consideration.

On receipt, full grant applications are sent to three external referees, one of whom is from outside the UK if possible. The grant administrator will distribute the papers and collate responses, which are then considered by the MAB. Projects are rated according to their quality and relevance to the Retina UK research strategy.

The referees' reports and the reports from the MAB meeting are collated and prioritised by the Chair of the MAB prior to their presentation to the Board of Trustees.

A final funding decision on the basis of the peer review, MAB recommendations, and the availability of finance, is taken after open discussion by the Board of Trustees. Such decisions will only be taken as agenda items for which full papers have been circulate prior to the Trustees' meeting.

### **Support for the Executive Team**

Retina UK office staff often need scientific advice, particularly in matters relating to grant administration and fundraising applications. In order to ensure needs are addressed efficiently:

- Staff members will send an email to both medical trustees outlining the issue.

- Ideally the two will confer and respond with a proposed way forward.
- If no response is received within two working days staff will make contact by telephone and take advice or instructions from whichever of the medical trustees is available.
- To answer some enquiries the medical trustee may refer the matter to another clinician or scientist (not necessarily a member of the MAB) which the Executive can trust to provide a reliable response.

## Appendix III: Restricted Funding

Retina UK recognises that members of the inherited sight loss community or biotechnology industry may wish to provide funding, via Retina UK, to answer a specific research question or to support the work of a particular researcher.

In order to maintain high standards of scientific integrity in relation to the research we fund and facilitate, as of 1<sup>st</sup> January 2019, Retina UK will adhere to the following processes when considering these requests.

- Retina UK will move from one annual open call for proposals to three open calls per year (preliminary application deadlines: 1<sup>st</sup> September (publicised), 1<sup>st</sup> February, 1<sup>st</sup> June). There will also be an option to run a call for a specific research question concurrent with any of these open calls.
- Researchers who have generated a project proposal can apply for funding from Retina UK's core\* funds via any of the three open calls. These proposals will follow our current peer review pathway: they will be assessed and scored by the MAB on the basis of scientific merit and feasibility, and those suitable for funding will be considered by the Board of Trustees in the context of their MAB ranking and available budget.
- Should a donor wish to fund the work of a specific researcher, that researcher will be invited to apply to Retina UK at the next open call, which may, at the discretion of Retina UK, be publicised to the research community and will accept applications on any topic in line with our overall strategy. The application will follow our current peer review process: it will be judged by the MAB on scientific merit and feasibility alone and, if deemed to

be suitable for funding, will then be considered by the Board of Trustees alongside other applications in that call. The Board will consider the application in the context of MAB ranking, the proportion of the project cost that will be covered by the donor(s) and Retina UK's available core funds. Where the donor is not covering the full amount, the Board will always prioritise the use of core funds for competitive projects with the highest MAB ranking and greatest translational potential for our community.

Should the MAB deem the proposal to be of an insufficient standard, Retina UK will not facilitate funding, regardless of the proportion of costs contributed by the donor. However, the Principal Investigator may amend the application and resubmit at a future call, when it will be assessed as a new application.

- Should a donor wish to commission Retina UK to conduct a call for proposals in response to a specific research question, the entire cost of the final approved project must be met in full by the donor. The donor must also commit to reimbursing the cost of any Retina UK resource employed in administration or fundraising.

The research question must be approved by the MAB Chair or Vice-Chair and the available budget will be clearly stated in the call. This specific call will be timed to run in concurrence with the next Retina UK open call. The MAB will assess applications to the specific call on the basis of scientific merit and rank those that are suitable for funding, completely separately from any applications received in the open call. Suitable applications will be then be presented to the Board for their consideration. Should none of the proposals meet the high standards of scientific merit that we expect, Retina UK will not facilitate the project.

- Should a donor wish to fully fund the purchase of an item of equipment by an IRD research group, separately to any project funding, then Retina UK will consider facilitating this at our discretion.

Under no circumstances will Retina UK accept restricted funds for a project prior to it being approved via the processes outlined above.

Retina UK is also funding pioneering research carried out at specific centres of excellence in the UK with an AMRC compliant process in place to ensure that the research meets high standards of scientific merit.

\*Core funds refers to any Retina UK funds generated from donations that are not restricted to a specific project, researcher or research question.

## Appendix IV: Project cost- and no-cost extensions

Retina UK realises that grantees will occasionally encounter situations that require the timeline of a project to be extended, with or without additional costs incurred.

### **No-cost extensions**

- Bridge funding or funding that has been granted in extreme circumstances to cover a shortfall will not be extended. This funding must be used within the period originally set out and for the purposes originally agreed. Any funds not invoiced within 3 months of the end of the agreed period will be retained by Retina UK.
- Requests for a no-cost extension must be accompanied by an explanation of the circumstances leading to the request. Requests will be considered in exceptional circumstances, such as HR issues.
- Grantees may make one no-cost extension request per year of the grant.
- The total extension over the life of the grant must not exceed 12 months. Any funds not invoiced within 3 months of the end of the agreed extended period will be retained by Retina UK.

### **Cost extensions**

- Requests for cost extensions must be accompanied by a detailed explanation of the circumstances necessitating further funding. This will be reviewed by the Chair of the Retina UK Medical Advisory Board, our Chief Executive and our Treasurer.
- Only one cost extension will be considered in the course of a grant.
- Extended funding must be used within the agreed extended period. Any funds not invoiced within 3 months

of the end of the agreed extended period will be retained by Retina UK.

- The total extension over the life of the grant must not exceed 12 months. Any funds not invoiced within 3 months of the end of the agreed extended period will be retained by Retina UK.

Appendix V: Sight Loss and Visions Priority Setting Partnership - Priorities for Inherited Retinal Diseases  
Below are Retina UK's priorities selected from SLVPSP document. All research must address one or more of these.

**Priorities:**

1. Can a treatment to slow down progression or reverse sight loss in inherited retinal diseases be developed?
  - How can sight loss be treated in people with an inherited retinal disease?
  - Is it possible to determine which inherited retinal diseases are likely to be treatable with gene therapy?
  - Can a stem cell therapy stop progression of sight loss and restore sight for inherited retinal diseases and for syndromes associated with RP, such as Usher and Alström?
  - Will gene therapy stop the progression of sight loss and reverse sight loss in inherited retinal diseases and in syndromes associated with RP, such as Usher and Alström?
    - What is the likelihood that computerised artificial eyes/retinal implants can restore sight loss due to inherited retinal disease?
  - Are there any potential long term risks associated with gene therapy for inherited retinal diseases?
  - Are there any potential long term risks associated with potential stem cell therapies for inherited retinal diseases?
  - Could a treatment in the form of eye drops be developed for inherited retinal diseases?

2. How can sight loss be prevented in an individual with inherited retinal disease?
3. Is a genetic (molecular) diagnosis possible for all inherited retinal diseases?
  - Is access to genetic testing available for all inherited retinal diseases?
4. What factors affect the progression of sight loss in inherited retinal diseases?
  - Why do some patients with a genetic mutation not develop the disease?
  - Can the rate of sight loss for people with RP be predicted?
  - How much is known about the long term prognosis (natural history) for inherited retinal diseases and is it related to the genotype?
  - Can dietary measures, nutritional supplements, vitamins, complementary therapies or lifestyle changes affect the progression of sight loss in inherited retinal diseases?
  - Can lifestyle or dietary factors trigger or prevent the onset of sight loss in RP?
5. What causes sight loss in inherited retinal diseases?
6. What is the most effective way to support patients with inherited retinal disease?
  - What types of glasses/lenses can be beneficial for people with RP?
  - What is the likelihood that the use of sunglasses from an early age can prevent sight loss in RP?
  - Once diagnosed should patients with an inherited retinal disease be regularly seen by an ophthalmologist even when there are no current treatments?

6. Can the diagnosis of inherited retinal diseases be refined so that individuals can be given a clearer idea about their specific condition and how it is likely to progress?
8. What is the relationship between sight loss and mental health for people with inherited retinal diseases?
9. With regard to inherited retinal diseases what is the role of pre-natal and pre-implantation diagnosis in helping parents make informed choices?