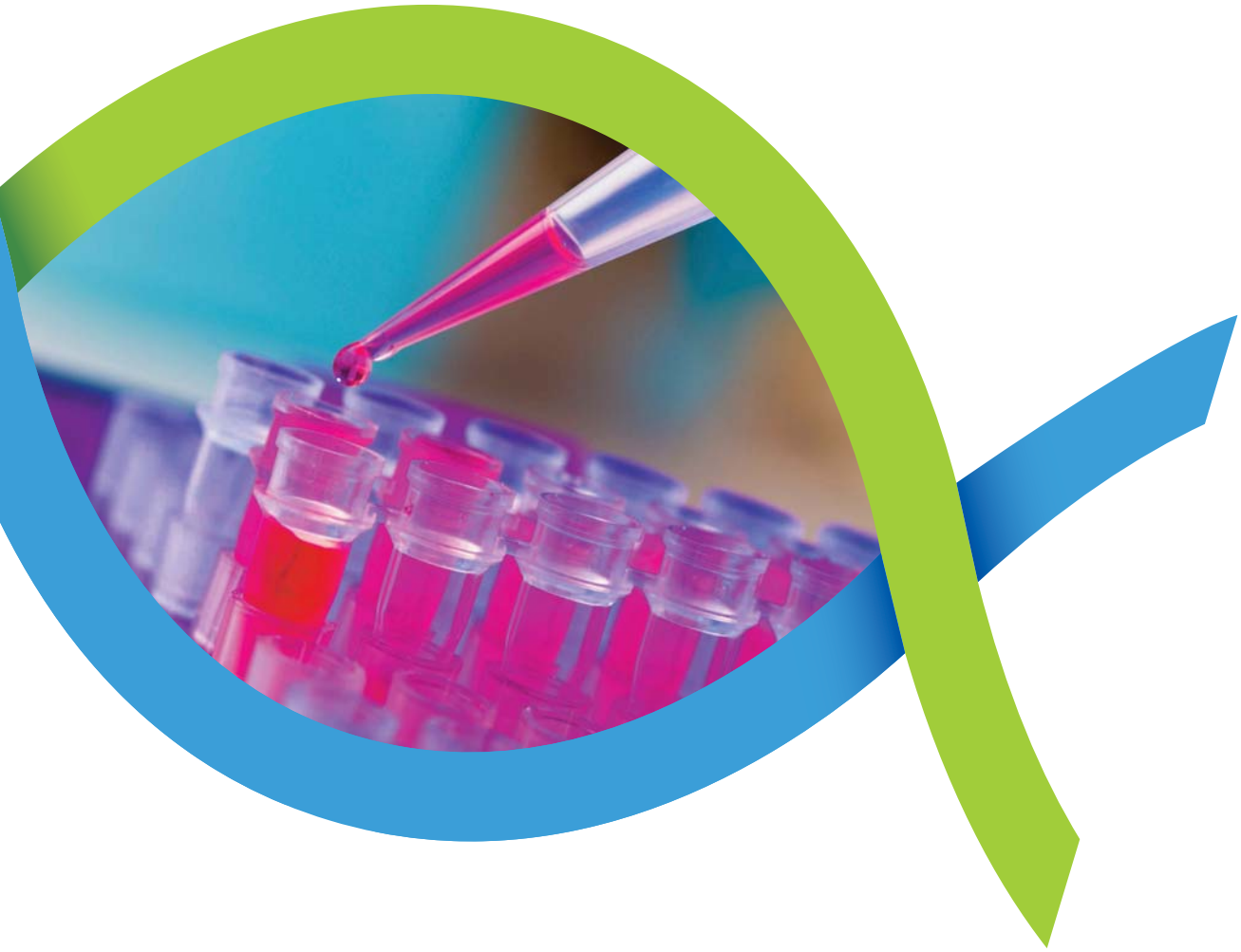
FIGHTING BLINDNESS

Registered
Charity No.1153851

Research Strategy



RP Fighting Blindness is a Charitable Incorporated Organisation (CIO), registered charity number 1153851.

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Our Mission

Our Research Mission

To stimulate and support high quality medical research with the aim of increasing scientific understanding of inherited retinal dystrophies (IRDs), 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 inherited 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

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

RP Fighting Blindness has since evolved into a respected medical research charity, funding cutting edge research into the causes and potential treatments for the condition, and is a nationwide organisation providing support and information for those affected. In recent years the charity has funded £500,000 - £700,000 of research per annum and 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.

For more information about our past research, please visit www.rpfightingblindness.org.uk

Research aims and objectives

RP Fighting Blindness encourages high quality research applications in any area of research related to retinal dystrophies and priority will be given to projects with promise for progressing the development of treatment strategies or slowing down the advancement of sight loss. This includes basic laboratory research investigating disease mechanisms, preclinical studies in animal models and genotyping and phenotypic investigation of patient populations as a prelude to clinical trials.
RP Fighting Blindness is focussed on understanding the causes of inherited 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 causes, 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 the identified areas of importance including but not limited to:
    - Genetic Testing
    - Artificial Retina
    - Gene Therapy
    - Protein Chemistry
    - Stem Cell biology
    - Cell Biology
    - Phenotyping
    - Molecular Genetics
    - Nutritional and environmental studies
- To support the early detection of RP and inherited retinal dystrophies
- To encourage and stimulate the dissemination of research results and relevant collaborations to avoid duplication of work and drive advancement of treatments, diagnostic tools and prevention aids
- To maintain a robust and accurate assessment process, including relevant scientific recommendation and peer review analysis.

Research Aims and Objectives

To realise these aims RP Fighting Blindness will:

- Fund innovative breakthrough research
- Focus on research specifically concerned with the diagnosis, treatment and prevention of inherited retinal dystrophies
- Strive for standard classification of inherited retinal dystrophies diagnosis and register those patients
- 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
- Facilitate effective communication between patients, scientists and clinicians to promote mutual understanding and potential participation in studies, where appropriate.

Appendix I

Criteria and Types of Funding

Fundamental Criteria

- High quality research project involving respected and experienced research leaders.
- Relevant to RP Fighting Blindness research objectives and aims as set out previously in this document.
- Value for money.
- Advances the objectives of RP Fighting Blindness. (This may include projects that are helpful for fundraising purposes).
- The research must be legal, and subsequent resulting treatment recommendations also legalised.
- Projects considered must be between one year and five years in duration.
- The application must be made by a professional regulatory or statutory body (or equivalent) accredited program.

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. RP Fighting Blindness may support bright young scientists who are starting their careers in relevant research.
- Where applicants have received previous grants from RP Fighting Blindness there must have been good outcomes and publications related to the funded project.
- Priority will be for project grants and MD / PhD studentships. Programme grants or fellowships or grants for equipment may be approved if finance allows.
- RP Fighting Blindness 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.
- There are no geographic location restrictions on applications.
- That research is appropriately publicised.
- Require that grant holders manage intellectual property that arise and, where appropriate, actively work towards facilitating its future advancement or clinical trials.

Appendix I

- 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

Grant Process and Administration

The Medical Advisory Board (MAB) operates according to the Protocol below:

- Project applications are invited on annual basis, but also (when funding allows) ad hoc calls will be made. 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 a six-monthly 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 RP Fighting Blindness.

Summary of elements funded:

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

Appendix I

Elements not included:

- 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.
- There is a dedicated administrator who will be assigned to support the project leader (including compiling of reports etc).
- Six monthly reports will be expected by the Board of Trustees, and satisfactory completion and submission is needed to guarantee continuation of funding.
- The RP Fighting Blindness 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 and selection process.

Appendix II

Medical Advisory Board (MAB) 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 every six months as publicised on the RP Fighting Blindness website and, if appropriate, in medical and scientific publications.
  This is summarised as:
    - Submission deadline last Friday of September, final decision at trustees meeting in April
- To meet on a regular basis 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 RP Fighting Blindness relating to clinical and scientific aspects of retinal disease.
- To regularly review this policy / protocol document and make recommendations to the Board of Trustees concerning research priorities.
- To assist and support the RP Fighting Blindness office in matters related to grant administration where a scientific input is required.
- To assist and support RP Fighting Blindness fundraisers with scientific inputs to donor applications.
- To provide RP Fighting Blindness 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 RP 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.

Appendix II

Membership of the MAB

All Medical Advisory Board (MAB) members are appointed by the Board of Trustees, based on a recommendation by 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.

MAB members may be RP Fighting Blindness grant holders but will excuse themselves from the MAB's discussions of their grants and projects and leave the room. Where closely related teams submit applications' all members of the MAB are expected to declare such relationships to the MAB and to the Board of Trustees.

The charity accepts that given the specialist nature of our work and the need to engage with the very best scientists in a limited field that such interests will exist on the MAB.

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 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. It is the full application that must be received by the deadline.

Appendix II

Full grant applications will be peer reviewed (see below) by three independent referees chosen by the Chair of the Medical Advisory Board with advice from other members as necessary. Applications, peer reviews and twice-annual progress reports will be circulated to Medical Advisory Board members at least 14 days in advance of the yearly Medical Advisory Board meeting. If it proves impossible to secure three independent reviews the Medical Advisory Board will review internally along with whatever reviews are available.

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

The MAB meeting will be held 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 two meetings per year, 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 RP Fighting Blindness Board of Trustees. The Chief Executive or another member of the Executive Team will be invited to attend such meetings.

Peer Review Process

All RP Fighting Blindness grant applications are subject to peer review. Referees may be selected from the MAB or from other relevant experts in the field of the application under consideration.

On receipt, full grant applications are sent to three 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 RP Fighting Blindness research strategy.

Appendix II

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 circulated prior to the Trustees' meeting.

- 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

Sight Loss and Visions Priority Setting Partnership (SLVPSP) - Priorities for Inherited Retinal Diseases

Below are RP Fighting Blindness's priorities selected from the SLVPSP document. All research must address one or more of these priorities. (This list is not exhaustive)

Conditions including:

- Achromatopsia
- Best Disease
- Choroideremia
- Cone Dystrophies
- Leber's Congenital Amaurosis
- Retinal Dystrophy
- Retinitis Pigmentosa (RP)
- Sorsby Macular Dystrophy
- Stargardt's Disease
- Usher Syndrome

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?

7. 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?