



PCD Research

PCDresearch.org

PCD Research – Scientific Strategy January 2023

1. Why PCD Research Funds Research

PCD Research is committed to improving outcomes for people with Primary Ciliary Dyskinesia (PCD) and believe the best way to do this is to fund high impact and collaborative research.

2. PCD Research

PCD Research is the only charity worldwide with the sole focus of supporting research focused on PCD. Over 50% of research funded in the UK is supported by charitable grants. The work of PCD Research is vital to ensure the rapid advancement of targeted research into PCD both in the UK, and globally, in order to improve outcomes for people with PCD.

3. PCD Research Scientific Priorities

PCD is a multisystem disorder, primarily caused by the abnormal or absent movement of motile cilia. Motile cilia are the microscopic hairs that beat in the airways and sinuses, clearing out secretions and infections. In PCD, the cilia are abnormal and unable to move in the usual way, such that secretions and infections affect the lungs, sinuses, ears and nose. Cilia are also important for the propulsion of sperm, so fertility is frequently affected too. PCD is associated with infertility and subfertility, with 75% of males thought to experience infertility, and high female infertility rates (63%) reported in some studies.

People with PCD experience a range of symptoms due to the multi-system nature of the disorder, whereby motile cilia are paramount to numerous processes within the body. In addition, new research suggests that in a subset of people with microtubular PCD lung function is more severely affected.

Although these symptoms are important, particularly for patient reported outcomes, progressive decline in lung function has the most profound effects on mortality and morbidity.

PCD is a life-altering and life shortening condition requiring intensive daily treatments and currently lacking disease-specific therapies. Often compared to Cystic Fibrosis, the lung function and disease burden of some people with PCD has been shown to be comparatively worse than in CF. Progressive respiratory failure in PCD is associated with poor quality of life, substantive morbidity and in some cases requires lung transplantation.

Therefore, during the peer review process by our independent Scientific Advisory Panel (SAP), importance will be placed on PCD Research's ultimate goal of improved respiratory outcomes as the most impactful way to improve overall outcome in PCD. An example of a potential route to improved respiratory outcome would be establishing functional restoration of motile cilia, such that the symptoms of PCD are reduced, and the progressive decline in lung function is halted. It is important that proposals demonstrate how the research may lead to patient benefit according to the objectives of PCD Research.

PCD Research will consider supporting all stages of research from laboratory projects, translational research, pilot projects to clinical trials. We will grant funding for research that promotes understanding of the cause, cure and care of PCD. While PCD Research accepts that more understanding of cilia is important, cellular biology that is not ultimately focused upon a route to cure of PCD, is beyond the resources of PCD Research. Epidemiology and quality of life-based projects will not be considered.

4. PCD Research Funding Strategy

The types of grants that PCD Research will be able to offer, will depend on donations and collaboration opportunities. PCD Research anticipates offering a range of grant types including, but not limited to, small projects, post-doctoral students, PhD fellowships, and more strategic longer term grants.

PCD Research will consider commit to funding long-term grants to support clinical centres with sample collection for use in active research projects. Grant applications will be reviewed as per normal practice, however, in order to manage the continuous review and recommendations for the continuation of funding, PCD Research have implemented the following:

- i. Progress and impact of these grants will be measured yearly
- ii. The progress report is available on the PCD Research website and will be used as part of the review process
- iii. PCD Research may conduct audits of the centres to measure progress
- iv. Review of the centres and recommendations for the continuation or discontinuation of funding will be made by the SAP to the Board of Trustees

- v. The Board of Trustees will give final approval for the continuation of funding
- vi. Centres will be informed of the decision and feedback given on the review in a timely manner

5. Who PCD Research Will Fund

PCD Research places importance on maximising the impact of research for the advancement of understanding and curing PCD by:

1. Nurturing the careers of early-stage research staff with an interest in PCD research
2. Working with research teams to develop their research ideas through a pilot grant scheme
3. Supporting proposals that are likely to win support from larger funding bodies
4. Encouraging collaboration between research groups
5. Encouraging interdisciplinary teamwork and partnership
6. Facilitating access to primary tissue and clinical data

To be eligible to apply for PCD Research funding, the following criteria must be met:

a) Applicants and Grant Holders

Sole applicants or applicant supervisors must have:

- i. International standing as a research leader in the field
- ii. Experience of leading innovative and creative research
- iii. Track record of managing and training others

We will review sole applicant or lead applicant's:

- i. Research outputs and contributions to the research community
- ii. Previous contributions to, and plans for, developing team members and other researchers
- iii. Leadership and management skills, and how the applicant (s) plan to develop these during the award
- iv. Rationale for a team approach, team composition (including the applicant's approach to diversity, inclusion and career stage) and the contribution of each team member.
- v. Appropriate scientific or medical degree qualifications.

b) Research Proposal

To be competitive, the research proposal will be:

- i. High quality. It is well-designed, clear, supported by evidence and the proposed outcomes/outputs are feasible.
- ii. Bold. It aims to deliver a significant shift in understanding and/or it provides a significant advance over existing methodologies, conceptual frameworks, tools or techniques. It has the potential to stimulate new and innovative research.

- iii. Creative. The proposed approach is novel. It develops and tests new concepts, methods or technologies, or combines existing ideas and approaches in a new way.
- iv. Specific. Demonstrates the purpose is primarily for the benefit of understanding/advancing outcomes in PCD, rather than allied diseases.

c) Institution

All applicants must have sponsorship from one of the following types of institution:

- a. Higher education institution
- b. Research institute
- c. Non-academic healthcare organisation
- d. Not-for-profit organisation

6. Where PCD Research Will Fund Research

PCD research will potentially fund research globally. Locations will be limited to places where there is robust and ethical scientific regulation such that any research will be able to be performed in line with our Scientific Strategy, Funding Policy and Animal Research Policy and the World Health Organisation's Code of Ethics and Professional Conduct.

7. Funding Exceptions

PCD Research does not fund:

- Applicants that are applying for, hold, or employed on a research grant from the tobacco industry.
- Epidemiology and quality of life-based projects will not be considered.
- Projects and applicants that do not meet our funding criteria, as outlined in this document and the PCD Research Funding Policy.

8. PCD Research Grant Awards

PCD Research will periodically release funding calls, to alert the research community of grant opportunities. For each individual funding call, the timeline for application and peer review will be clearly defined.

9. Scientific Strategy Review

PCD Research will review the scientific strategy every 2 years.