



(A Charitable Incorporated Organisation Regulated by the UK Charities Commission)

**Registered Number
1197528**

**Annual Report and Accounts
Period ending
31st December 2022**

**Registered Address:
87 Belsize Lane
London NW3 5AU
info@PCDResearch.org**

1. Overview

This document provides the annual report and accounts of PCD Research CIO ("PCD Research", or the "charity"). The trustees present their report and accounts for its initial financial year, the period from its date of incorporation on 17th January 2022 to 31st December 2022.

1.1. Objectives and Principal Activities

The charity is registered and regulated by the UK Charity Commission. Its reference is 1197528.

The objectives of the charity are to advance the health of children and adults with primary ciliary dyskinesia (PCD) by supporting research into A) understanding PCD and B) novel therapeutic targets and therapies for PCD. The charity will carry out these objectives by funding pure scientific and applied/translational research for the public benefit of improving the health and outcome of people with PCD.

1.2. Trustees and Governance

PCD Research does not have any staff at the moment. Instead, it is governed by its board of trustees, who are dedicating their time and efforts on a pro bono basis. PCD Research was set up as a Charitable Incorporated Organisation. Its constitutional document includes provisions relating to the appointment of trustees. Each trustee is appointed for an initial term of three years, whereupon they can be reappointed.

The following persons served as trustees during the period ending on 31st December 2022:

Dr Harriet Holme - Chair of the Trustees - Appointed on 17th January 2022

Dr Gurhan Erturan - Trustee - Appointed on 17th January 2022

Natalie Gehl – Trustee - Appointed on 17th January 2022

Lucy Dixon – Trustee - Appointed on 17th January 2022, resigned on 24th April 2023

Subsequently, the following additional trustees were appointed:

Michelle Levene - Trustee - Appointed on 24th April 2023

Harriet Nowell-Smith - Trustee - Appointed on 24th April 2023

Oliver Burgel - Trustee - Appointed on 24th April 2023

The trustees were approached and appointed with the aim of establishing a board with diverse professional experience at senior level as well as direct patient experience and impact of PCD on family life. At present, the range of professional expertise covers clinical medicine, research (scientific, translational and medical), drug development strategy and development in advanced therapeutics, rare diseases, public and international law, fundraising and finance. Two of the trustees are parents of a children with PCD.

The charity's key policy documents, such as Conflict of Interest Policy, Funding Policy, Animal Research Policy Expense Policy and Research Strategy are available on request by contacting info@PCDResearch.org.

The charity's address is 87 Belsize Lane, London NW3 5AU.

2. Trustees' Report

PCD Research is a medical research charity that was incorporated and registered with the UK Charity's Commission on 17th January 2022. The aims of PCD Research include improved treatment options for people with PCD by funding research and improving the standard of care. It is the only charity worldwide funding research towards finding a cure for PCD. PCD Research also uses conceptual models from the field of oncology to advance research into better treatments.

2.1. What is Primary Ciliary Dyskinesia

Primary ciliary dyskinesia is a genetic condition that affects approximately one in 7,500 people. Mutations in approximately 50 genes have been found to cause PCD. This leads to a range of severity. PCD leads to permanent damage of the lungs, and for people with the most severe disease, this happens decades earlier than those with milder disease. One in 20 people are carriers with one affected copy of a gene that causes PCD. Leaders in the field think that carriers might have a separate set of symptoms that overlap with difficult to treat asthma.

Motile cilia are like microscopic hairs that beat throughout the body, including 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 commonly affected as well. It is likely that PCD affects other pathways in the body in ways that are not yet understood.

2.2. Treatment and Outcome

PCD is a life altering and life shortening condition. Children with PCD have been found to have worse lung function than those with cystic fibrosis (CF) but are unable to access the same standard of care in the UK. In the case of CF, there has been an active program of research that has led to breakthrough drugs that almost cure CF. Sadly these drugs are not suitable for people with PCD.

At present the respiratory aspect of PCD is managed by a brutal regimen of chest physiotherapy to try to prevent and slow lung function decline. There are no dedicated treatments for PCD, instead current treatments have been borrowed from experience with people with CF. Physiotherapy is supplemented with frequent courses of antibiotics to treat and reduce episodes of pneumonia. This is a significant burden on people with PCD and their families.

People with PCD face dramatic challenges from living with the disease. Treatment causes an enormous burden in terms of the hours of daily chest physiotherapy needed to clear the lungs of mucus, using methods borrowed from a mechanistically distinct disease (cystic fibrosis) without evidence of efficacy in PCD. In addition, patients are subject to frequent courses of antibiotics in an attempt to reduce the rate of lung function decline. Some people with PCD will still need a lung transplant.

While children and adults with PCD may look healthy, PCD is a progressive disease, where lung function declines over time. At present there are no medications that can stop this decline or restore cilia function. There are no NICE guidelines. The Commissioned Service Providers are currently developing a Standard of Care.

2.3. Formation of the Charity and Research Objectives

PCD Research was set up to advance the health of children and adults with primary ciliary dyskinesia (PCD) by supporting research into A) understanding PCD and B) novel therapeutic targets and

therapies for PCD. The charity will carry out these objectives by funding pure scientific and applied/translational research for the public benefit of improving the health and outcome of people with PCD.

2.4. Achievements of PCD Research during 2022

The key achievements of the charity during its first year after foundation can be summarised as follows:

- Establishment of the charity,
- Appointment of a world-class Scientific Advisory Panel (SAP),
- Development of a research strategy, and
- Establishment of successful fundraising and grant application process.

2.4.1. Appointment of a Scientific Advisory Panel

To ensure that the most promising research is funded, PCD Research is engaging with leading academics in the fields of PCD, CF, bronchiectasis, gene augmentation and gene editing. This has enabled PCD Research to form a Scientific Advisory Panel (SAP) with a range of experience, who are international and independent, to focus on development of novel therapies for PCD. The members of the SAP were appointed to robustly scrutinise grant applications, so that only the most promising research is funded. The SAP currently comprises 25 academics from top tier universities and teaching hospitals in the UK, US, Germany, Canada, Israel, France and Belgium.

Academics in the PCD Research community have been delighted to offer up their time to do this, and were excited at the possible future research opportunities PCD Research will facilitate. Heidi Bjornson-Purnell was appointed as the Chair of the SAP. Heidi currently works as a project manager at the Chan Zuckerberg Initiative (CZI) Rare As One, is a former barrister and the parent of two children with PCD.

2.4.2. Development of a Research Strategy

PCD Research's primary goal is to advance the health of children and adults with Primary Ciliary Dyskinesia (PCD). To meet this mission, our research strategy focuses on targeting the most severe disease phenotypes (inner dynein arm defects with microtubular disorganisation caused by loss of function in genes *CCDC39* and *CCDC40*) because these have the greatest chance of demonstrating efficacy and therefore attaining regulatory approval. This patient population is relatively easy to identify and their form of PCD is so severe that they are likely to be willing to participate in time-consuming and invasive medical trials. We envisage this as a proof of concept stage, such that any novel therapy could then simply be readily adapted for other genes and variants that cause PCD.

PCD Research was founded to support the development of disease modifying or curative therapies for PCD. We support the strategy of prioritising the development of therapies that:

- will have the greatest impact for all patients with PCD within in the shortest period of time;
- are likely to have the greatest success through the regulatory process;
are likely to justify the use of public money for research by having the potential to deliver the greatest savings for publicly funded health care systems (not just in England) by postponing or removing the need for lung transplants.

Development of novel therapies in the most severe disease phenotype would help those people who experience the most severe disease and are most at risk of dying prematurely.

2.4.3. Establishment of Successful Fundraising and Grant Application Process

During its first year of operation, PCD Research established a fundraising routine and was successful in securing two scientific collaborations.

In 2022 PCD Research raised £153,776.27 through donations. This was raised from a small number of substantial individual donations and various community fundraising efforts. As Chair of PCD Research I would like to personally thank the Brooks family for their substantial fundraising efforts as “100G for PCD”, together with donations from Matt Holme, and the Mather Family Charitable Trust.

The scientific collaborations are described below:

- **National Facility for Mouse Genetics Network “Patient-led functional genomics” Cluster, Mary Lyon Centre, Harwell**

In collaboration with Professor Mill, co-lead of the UKRI MRC National Mouse Genetics Network cluster ‘Congenital anomalies: patient-led functional genomics, the Medical Research Council (MRC) funded National Facility for Mouse Genetics Network (NMGH) “Patient-led functional genomics” cluster at the Mary Lyon Centre, Harwell, has agreed to fund an inducible model of PCD caused by loss of function of gene *CCDC39*. PCD Research was instrumental in negotiating and advocating for this patient-derived PCD mouse model. We believe this will be valuable for the research community and will provide the opportunity for future *in vivo* validation of novel therapeutics, together with understanding delivery challenges and safety studies.

- **Nucleic Acid Therapeutic Accelerator (NATA) Collaboration**

PCD Research will collaborate with the Medical Research Council (MRC) funded Nucleic Acid Therapy Accelerator (NATA) to jointly fund a two-year post-doctoral position. PCD Research will contribute one third (£83,333) of the total cost (£250,000) of this project.

The scope of this project will be based on the optimisation and delivery of nucleic acid therapies in PCD. Development and optimisation of mRNA therapy that has the potential to restore ciliary function in air-liquid interface cell culture (ALI), which allow scientists to generate stable and functional in vitro 3D human airway cell models that closely mimic respiratory tract epithelia. A grant call for applicants to determine the exact nature of the project and supervisors occurred in the first quarter of 2023.

The additional benefit to this project is the added significant potential for the researchers to see if the therapy works in a mouse model of PCD that will go into production in Q3 2023 at the MRC funded National Mouse Genetics Network Congenital Anomalies Cluster (loss of function of the *CCDC39* gene). This research will provide vital proof of concept for future research targeting different genes causing PCD.

2.4.4. Other Outreach

A website for PCD Research was built in January 2022 that details the mission, shares information about the charity, about the epidemiology of PCD, the SAP and provides information for researchers. @PCD_research and @PCDresearch have been set up on Instagram/Facebook and Twitter respectively, to enable communication of our mission, goals and progress with the patient population, academics and future commercial partners.

PCD Research has also founded links with other organisations representing patients with PCD, both PCD Support UK and internationally the PCD Foundation, USA. Dr Harriet Holme was invited to

highlight the work of PCD Research at PCD Support UK's annual medical board meeting with both an oral presentation and poster.

Dr Harriet Holme represented PCD Research at the EMBO Cilia2022 conference held in Cologne in October 2022. This was a valuable meeting to talk with academics from around the world about the latest research in PCD, roadblocks and how to successfully navigate round these.

2.4.5. Outlook

While the charity had early successes in fund raising and grant application processes, it did not incur substantial expenses during the year of its foundation. This is expected to change during 2023 as the NATA grant will be disbursed through the charity from the beginning of the 2023/24 academic year.

The SAP held its inaugural meeting in March 2023 to peer-review the applications for project and supervision of the research collaboration with NATA. We anticipate that the process of advertising/appointing a post-doctorate position at UCL to start in Q3/4 2023.

PCD Research applied, and was granted membership in February 2023, to the Association of Medical Research Charities (AMRC). AMRC membership is the hallmark of quality research funding for medical research charities. Attaining membership recognises the processes we have put in place to ensure PCD Research only funds research of the highest standards through the SAP peer-review process. We will continue to expand and establish further academic links with the research community and commercial links with interested parties.

PCD Research will continue to build community links with schools for ground roots awareness raising and fundraising and consider our wider strategy for fundraising, while continuing to engage with potential cooperate donors. PCD Research will also seek to form and strengthen links with other charities supporting rare disease such as Genetic Alliance (which PCD Research joined in June 2023) and RareBeacon.

This report was approved by the trustees on 15 August 2023 and signed on their behalf.



Dr Harriet Holme
Chair

3. Statement of Trustees' Responsibilities

The Trustees are responsible for preparing the annual report and the financial statements in accordance with applicable laws and regulations.

UK Charity law (Charities Act 2011 and subsequent amendments) requires the Trustees to prepare accounts for each financial year. The accounts have been prepared on a receipts and payments basis as provided for under section 133 of the Charities Act 2011. PCD Research is a smaller charity for the purpose of reporting.

Under charity law the Trustees must not approve the accounts unless they are satisfied that they give a true and fair view of the state of affairs of the charity and of the profit or loss of the charity for that period. When preparing these accounts, the Trustees:

- selected suitable accounting policies and applied them consistently;
- made judgements and estimates that are reasonable and prudent;
- prepared the financial statements on the going concern basis.

The Trustees are responsible for keeping adequate accounting records that are sufficient to show and explain the charity's transactions and disclose with reasonable accuracy at any time the financial position of the charity and enable them to ensure that the accounts comply with applicable regulations. They are also responsible for safeguarding the assets of the charity and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

This report was approved by the trustees on 15 August 2023 and signed on their behalf.



Dr Harriet Holme
Chair

4. Financial Review

4.1. Receipts and Payments for the period from 17th January 2022 to 31st December 2022

Notes	Unrestricted	Restricted	Endowment	Total 2022
	£	£	£	£
Receipts				
- Donations	153,776.27	-	-	153,776.27
- Charitable activities	-	-	-	-
- Investments	-	-	-	-
- Other	-	-	-	-
Total Receipts	153,776.27	-	-	153,776.27
Payments				
- Raising funds	-2,240.00	-	-	-2,240.00
- Charitable Activities	-228.18	-	-	-228.18
- Other	-	-	-	-
Total Payments	-2,468.18	-	-	-2,468.18
Net Income	151,308.09	-	-	151,308.09
Transfer of Funds	-	-	-	-
Revaluation of Fixed Assets	-	-	-	-
Other Gains / Losses	-	-	-	-
Net Movement in Funds	151,308.09	-	-	151,308.09
Balances Carried Forward at 17st Jan 2022	-	-	-	-
Balances Carried Forward at 31st December 2022	151,308.09	-	-	151,308.09

During the period ending December 2022 the charity recorded receipts of £153,776.27. The largest donation was made by an individual donor for the amount of £100,000. The remaining funds of £53,776.27 were raised from about 60 individual donations.

As the charity was in the process of commencing its activities, no grants were made during the financial year 2022. The payments made during the financial year were £2,468.18. These consisted of minor administrative expenses of £228.18 incurred in conjunction with operating the charity and £2,240 incurred for securing places for the 2023 London Charities Half Marathon that raised approximately £6,000.

4.2. Statement of Assets and Liabilities as at 31st December 2022

	Jan 2022	Dec 2022
	£	£
Fixed Assets		
Intangible Assets	-	-
Tangible Assets	-	-
Heritage Assets	-	-
Investments	-	-
Total Fixed Assets	-	-
Current Assets		
Stocks	-	-
Debtors	-	-
Investments	-	-
Cash at Bank	-	151,308.09
Total Current Assets	-	151,308.09
Liabilities		
Creditors: Amounts Falling Due Within One Year	-	-
Net Current Assets	-	151,308.09
Total Assets Less Current Liabilities		151,308.09
Creditors: Amounts Falling Due Within More Than One Year	-	-
Provision for Liabilities		
Net Assets	-	151,308.09
Capital and Reserves		
Unrestricted Funds	-	151,308.09
Restricted Funds	-	-
Endowment Funds	-	-
Total Charity Funds	-	151,308.09

As of 31st December 2022, the only asset owned by the charity consisted of cash held on its bank account. The charity has no financial or other liabilities.

As a result of the fundraising activities by the charity exceeding the expenses incurred, the charity's own funds at 31st December 2022 amounted to £151,308.09.



Harriet Holme
Chair

Approved by the trustees on 15 August 2023.

4.3. Cash-Flow Statement as at 31st December 2022

	Period ending 31st December 2022
Opening Cash as of 17th January 2022	0
Cash receipts during the year	153,776.27
Payments made during the year	-2,468.18
Net Movement of Funds	151,308.09
Closing Cash as of 31st December 2022	151,308.09

The charity's cash position evolved strongly as a result of the strong inflow from donations exceeding the administrative expenses incurred during the financial year. As a result, the year-end cash position in its bank account amounted to £151,308.09. This situation is expected to change during 2023 as the charity will begin to move towards funding its first research projects.

5. Notes to the Financial Statements for the period from 17th January 2022 to 31st December 2022

5.1. Accounting Policies

Basis of preparation

The accounts have been prepared on a receipts and payments basis as provided for under section 133 of the Charities Act 2011. PCD Research is a smaller charity for the purpose of reporting.

The charity is a Charitable Incorporated Organisation and does not have any subsidiaries or branches.

Recognition of Income

All forms of income are recognised on a cash basis at the point when the charity receives funds into its bank account.

Recognition of Expenses

All expenditures are accounted for on a payment basis and are recognised at the point where funds leave the charity's bank account.

5.2. Statement of Funds

	17 st January 2022	Receipts	Payments	31 st December 2022
	£	£	£	£
General Unrestricted Funds		153,776.27	-2,468.18	151,308.09
Designated Unrestricted Funds	0	0	0	0
Total Unrestricted Funds	0	153,776.27	-2,468.18	151,308.09
 Total Restricted Funds	 0	 0	 0	 0
 Total Endowment Funds	 0	 0	 0	 0
 Total Funds	 0	 153,776.27	 -2,468.18	 151,308.09

As of 31st December 2022, all funds represented as General Unrestricted Funds. These were not earmarked for a specific purpose. During the financial year 2022, the charity held neither restricted funds nor endowment funds. There were no transfers between any classes of funds during the year.

5.3. Independent Examination

As the 2022 annual receipts of the charity exceeded £25,000 an independent examiner was appointed to provide independent assurance that the charity's money has been appropriately accounted for. The independent examiner has waived any fees for his services.

5.4. Trustees Remuneration and Expenses

During the period ending on 31st December 2022 none of the trustees received any remuneration or benefits from an employment with the charity. In addition, no trustee expenses were incurred.

5.5. Related Party Transactions

During the period ending on 31st December 2022 the charity received a £25 donation from a trustee as part of the account opening process with the charity's account bank.

5.6. Reserves Policy

It is the charity's aim to hold reserves so that it can be confident of its financial position and can meet its financial obligations at any point. The charity's current financial obligations are of a discretionary nature.

The charity's reserves policy takes into account that it may commit to fund research expenditures over the medium term whilst recognising that there may be a level of volatility in its income due to the inherent uncertainty of fundraising activities. The trustees have therefore decided that PCD Research will not enter into financial commitments with third parties unless it has secured prior funding. The charity will hold sufficient funds in reserves to ensure that it can meet any contractual commitment to funding future research and clinical projects.

Notwithstanding the above, given the lack of data points on fundraising and a normalised level of expenses, the trustees have also decided to keep a minimum reserve of £10,000 at all times earmarked for any contingencies.

5.7. Guarantees and Secured Debts

As of 31st December 2022, no guarantees were given by PCD Research. No debts are outstanding as of the date of statement of assets and liabilities.

5.8. Subsequent Events

In December 2022 PCD Research was notified that its application for a £250,000 research grant in collaboration with the Medical Research Council (MRC) funded Nucleic Acid Therapy Accelerator (NATA) was successful. PCD Research and NATA will jointly fund a two-year post-doctoral position. PCD Research will contribute one third (£83,333) towards the budget, which is well within its available resources. The grant will be administered by PCD Research and the Scientific Advisory Panel subsequently decided to award the funds to a project team lead by Prof. Hart from University College, London. At the time of publication of this report, the charity was engaged in negotiating the terms of the contract with UCL.



Section A

Independent Examiner's Report

Report to the trustees/
members of

PCD Research

On accounts for the year
ended

Period ended 31 Dec 2022 (Charity
registered 17 Jan 2022)

Charity no
(if any)

1197528

Set out on pages

6-12 of the Annual Report and Accounts

(remember to include the page numbers of additional sheets)

Responsibilities and
basis of report

I report to the trustees on my examination of the accounts of the above charity ("the Trust") for the period from registration on 17 Jan 2022 to 31 Dec 2022.

As the charity trustees of the Trust, you are responsible for the preparation of the accounts in accordance with the requirements of the Charities Act 2011 ("the Act").

I report in respect of my examination of the Trust's accounts carried out under section 145 of the 2011 Act and in carrying out my examination, I have followed the applicable Directions given by the Charity Commission under section 145(5)(b) of the Act.

Independent
examiner's statement

I have completed my examination. I confirm that no material matters have come to my attention in connection with the examination which gives me cause to believe that in, any material respect:

- accounting records were not kept in accordance with section 130 of the Act or
- the accounts do not accord with the accounting records

I have no concerns and have come across no other matters in connection with the examination to which attention should be drawn in order to enable a proper understanding of the accounts to be reached.

Signed:

Date:

10 Oct 2023

Name:

Benjamin Greene

Relevant professional
qualification(s) or body
(if any):

Fellow of Institute of Chartered Accountants in England and Wales

Address:

21 Greville Park Road, Ashted, KT21 2QU