



**A Charitable Incorporated Organisation
Regulated by the Charity Commission for England and Wales**

**Registered Number
1197528**

**Annual Report and Accounts
Period ending
31st December 2023**

**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 second financial year, the period from 1st January 2023 to the 31st December 2023.

1.1. Objectives and Principal Activities

The charity is registered and regulated by the Charity Commission for England and Wales. 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 2023:

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

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

Monica Dawes – Trustee – Appointed 24th November 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 and finance. Two of the trustees are parents of a child 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

2. Annual Report of the Trustees

PCD Research is a medical research charity that was incorporated and registered with the Charity Commission for England and Wales 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.

2.1. What is Primary Ciliary Dyskinesia

Primary ciliary dyskinesia is a genetic condition that affects approximately 1 in 7,500 people. Mutations in more than 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, the damage 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 in the airways and sinuses to clear 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 and likely also both the fallopian tubes and endometrium leading to impaired transport of the oocyte and early embryo, 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)¹ 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 (CFTR modulators) that are widely recognised to be very effective for CF. Sadly these drugs are not suitable for people with PCD.

There are no dedicated treatments for PCD. Instead, current treatments for PCD have been borrowed from experience with people with CF. People with PCD and their family members face a significant burden and daily challenges from living with the disease. Daily treatment includes several hours of chest physiotherapy to try to clear mucus from the lungs, using methods borrowed from a mechanistically distinct disease (CF) without evidence of efficacy in PCD. In addition, patients are subject to frequent courses of antibiotics to treat frequent episodes of pneumonia. Some people with PCD will still progress to end stage respiratory failure and need a lung transplant. People with loss of function of the genes *CCDC39* and *CCDC40* are now widely acknowledged to have a significantly poorer outcome. The average length of survival post lung transplantation in people with PCD is 5.9 years².

While children and adults with PCD may appear healthy, PCD is a progressive disease, where lung function declines over time. At present there are no treatments that can stop or reduce this decline, nor restore cilia function. There are no NICE guidelines.

2.3. Achievements of PCD Research during 2023

The key achievements of the charity during its second year are:

¹Rubbo, B. *et al.* Clinical features and management of children with primary ciliary dyskinesia in England. *Arch Dis Child* **105**, 724–729 (2020).

²Marro M, *et al.* Lung Transplantation for Primary Ciliary Dyskinesia and Kartagener Syndrome: A Multicenter Study. *Transpl Int.* 2023 Feb 14;36:10819. doi: 10.3389/ti.2023.10819. PMID: 36865666; PMCID: PMC9970992.

- Inaugural meeting of the Scientific Advisory Panel to complete the first peer-review process.
- Becoming a Full Member of the Association of Medical Research Charities (AMRC) and joining other professional organisations.
- Supported an application for the £10 million LifeArc Rare Disease Translational Challenge bid for a national Rare Respiratory Centre, with PCD as one of the three disease exemplars.
- Consolidated governance and recruited pro bono legal support.
- Fundraising.
- Raised awareness in the UK Parliament and three relevant All Party Parliamentary Groups (APPG).

2.3.1. Inaugural Meeting of the Scientific Advisory Panel (SAP)

In March, the inaugural meeting of the SAP was held virtually to peer-review the grant applications from the first PCD Research grant call. This was to fund a two-year post-doctorate in collaboration with the Nucleic Acid Therapeutic Accelerator (NATA). Professor Hart's project was chosen by the SAP, out of a total of three high quality applicants.

The project will look to optimise lipid nanoparticle (LNP) technology encoding mRNA targeting loss of function of *CCDC39*, first in cellular models (air liquid interface culture – ALI culture). Successful LNP constructs will be taken forward to optimise nebulised delivery in healthy mice. The third phase will be to see if the LNP construct can functionally restore cilia function in a mouse model of loss of function of *CCDC39*.

The grant contracts between PCD Research and (1) NATA and (2) University College London are expected to be signed in 2024.

2.3.2. Joined Relevant Professional Organisations

The Association of Medical Research Charities (AMRC) is a membership organisation dedicated to supporting medical research charities in saving and improving lives through research and innovation. The AMRC ensures its members fund the best research by developing guides, providing training, and carrying out an audit of their funding processes. They unite and champion the sector, helping to drive positive change in the research and health landscape. In 2022, AMRC members invested almost £2 billion in UK research, equivalent to that funded by the NIHR and MRC combined.

In 2023, PCD Research became a member of the AMRC. AMRC membership is established as the hallmark for quality research funding. By granting membership, AMRC is recognising the processes put in place to ensure PCD Research funds research of the highest standards.

The clarity and independence of PCD Research's funding process was recognised by the AMRC, who congratulated the charity on having accomplished this from the outset. PCD Research will continue to expand and establish further academic links with the research community in the UK and overseas.

PCD Research seeks to form and strengthen links with other organisations supporting rare disease and therapeutic development and joined Genetic Alliance and the UK Bioindustry Association.

2.3.3. Supported an Application to the LifeArc Rare Disease Translational Challenge

The LifeArc Rare Disease Translational Challenge has the potential to transform the landscape of PCD by providing the first significant investment in the field. Dr Harriet Holme was invited by LifeArc to give the Keynote presentation at the launch event about her experience of the translational challenges in

rare disease. Dr Holme provided patient and public involvement and engagement (PPIE) and support for a £9.4 million National Rare Respiratory centre bid with PCD as one of the three disease exemplars.

2.3.4. Pro bono Legal Support

PCD Research has established contracts for pro bono advice from Pinsent Masons to (1) draw up PCD Research's standard grant terms and terms for the contracts between NATA, UCL and PCD Research and (2) provide advice for advertising campaigns to raise awareness about PCD.

2.3.5. Fundraising

PCD Research received over £40,000 in 2023 from a range of fundraising activities, including sponsorship from 16 runners who gave up their time to run in the London Landmarks Half Marathon generating over £6,000 of donations.

2.3.6. Raising Awareness to Improve the Standard of Care

Tulip Siddiq MP asked a Parliamentary question about PCD on the 1st September 2023, on behalf of PCD Research: "to ask the Secretary of State for Health and Social Care, what steps his Department is taking to ensure children with primary ciliary dyskinesia receive similar care to those with cystic fibrosis."

The following response was provided by Will Quince MP on the 11th September 2023³: "The Government is committed to improving the lives of those living with rare diseases such as primary ciliary dyskinesia. In 2021, the Government published the UK Rare Diseases Framework, providing a national vision for how to improve the lives of those living with rare diseases. The framework lists four priorities collaboratively developed with the rare disease community: helping patients get a final diagnosis faster; increasing awareness of rare diseases among healthcare professionals; better coordination of care; and improving access to specialist care, treatments and drugs. The framework committed to nation-specific action plans and England published its second Rare Diseases Action Plan in February this year. The framework and action plans are not disease specific, but aim to improve the lives of all people living with rare diseases."

Dr Holme worked with colleagues in Scotland and Wales to raise awareness in other parts of the UK.

2.3.7. Built relationships with relevant All Party Parliamentary Groups (APPG)

On the 7th September 2023, Dr Harriet Holme, Harriet Nowell-Smith and Oliver Burgel met Liz Twist MP, the chair of the APPG for Rare, Genetic and Undiagnosed Diseases. This meeting was to raise awareness about PCD, the lack of research funding and inequity of clinical care. Dr Harriet Holme was also invited to attend the APPG meeting on the 11th December 2023 and to give a short presentation on the challenges of research in rare disease. Dr Harriet Holme also consulted with Hugh McKinney, Policy adviser to the APPG for Respiratory Health and the secretariat for the APPG on Medical Research.

2.4. Industry engagement

The charity engaged with representatives of biotech and pharma to raise awareness about PCD, to demonstrate the ways PCD Research is involved in clearing translational roadblocks and to share information from a patient perspective.

³ <https://questions-statements.parliament.uk/written-questions/detail/2023-09-01/197023>

PCD Research, together with LifeArc and Weatherden, will host an industry day on 1st October 2024 entitled "Getting Cilia Moving." This event aims to accelerate development of disease modifying agents for PCD, by leveraging scientific advancements and progress made in other respiratory diseases. The charity aims to demonstrate to industry and funders the unmet need caused by PCD and progress made towards de-risking this space to change the value proposition, with applicability to the wider respiratory disease landscape.

2.5. Outreach

The website that was built for PCD Research was re-designed and updated during 2023. Interested members of the public are able to subscribe and receive updates by email from the charity.

@PCD_research on Instagram enables communication of our mission, goals and progress with more than 1000 followers. PCD Research is also active on LinkedIn.

PCD Research continues to have links with the PCD Foundation in the USA, PCD Support UK, the Ciliopathy Alliance, PCD Australia, and BEAT-PCD (based in the UK and Europe).

PCD Research was represented at the following meetings in 2023:

- Dr Harriet Holme:
 - Decentralising Science, Crick Institute (January).
 - GRC Physiological and Pathological Mechanisms of the Mucociliary System, Italy (February).
 - NATA symposium, Birmingham (May).
 - ARMC Festival of Partnerships, Wellcome Trust (October).
 - Westminster Health Forum policy conference, virtual (October).
 - 2nd World Orphan Drug Congress 2023, Barcelona (November).
 - Beacon – The London Rare Disease Showcase (November).
 - ABPI - Rare Disease event, London (December).
- Harriet Nowell-Smith and Michelle Levene:
 - BEAT-PCD meeting, Milan (September).
- Dr Harriet Holme, Gurhan Erturan, Harriet Nowell-Smith and Oliver Burgel
 - LifeArc Rare Disease Translational Challenge Launch (June).

2.6. Scientific Advisory Panel

To ensure that the most promising research is funded, PCD Research has dedicated time to 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 SAP were appointed to act as an advisory board to robustly scrutinise grant applications, so that only the most promising research is funded.

Katie Dexter, SAP patient representative stepped down on the 30th August 2023 to take the position of Chair of PCD Support UK. Ian Brooks was appointed on the 31st August 2023 as parent representative on the SAP. There are now 29 people on the SAP, including Ian Brooks and Heidi Bjornson-Pennell (chair).

2.7. Outlook

The charity committed to substantial expenses in 2023 when it awarded its first research grant. The grant is expected to be disbursed through the charity beginning in 2024, pending the successful negotiation of contractual terms between NATA, UCL and PCD Research.

The charity's other main expenses are reimbursing reasonable costs for attending meetings and conferences. It maintains appropriate reserves and has adequate funding to meet expected expenses during 2024.

This report was approved by the trustees on 24 May 2024 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 24 May 2024 and signed on their behalf.



Dr Harriet Holme
Chair

4. Financial Review

4.1. Receipts and Payments for the period from 1st January 2023 to 31st December 2023

Notes	Unrestricted	Restricted	Endowment	Total 2023
	£	£	£	£
Receipts				
- Donations	40,676.38	-	-	40,676.38
- Charitable activities	-	-	-	-
- Investments	125.67	-	-	-
- Other	-	-	-	-
Total Receipts	40,802.05	-	-	40,802.05
Payments				
- Raising funds	-231.00	-	-	-231.00
- Charitable Activities	-11,459.73	-	-	-11,459.73
- Other	-	-	-	-
Total Payments	-11,690.73	-	-	-11,690.73
Net Income	29,111.32	-	-	29,111.32
Transfer of Funds	-	-	-	-
Revaluation of Fixed Assets	-	-	-	-
Other Gains / Losses	-	-	-	-
Net Movement in Funds	29,111.32	-	-	29,111.32
Balances Carried Forward at 1 st Jan 2023	151,308.09	-	-	151,308.09
Balances Carried Forward at 31st December 2023	180,419.41	-	-	180,419.41

During the period ending December 2022 the charity recorded receipts of £40,802.05. The largest donation was made by a donor for the amount of £7,000. The remaining funds were raised from c. 300 small donations.

As mentioned in the annual report, the charity began to negotiate the legal terms of a contract to fund research at University College, London (UCL) in collaboration with Nucleic Acid Therapy Accelerator (NATA). The funding need for this grant is expected to be about £83,000. As the negotiations had not been completed by December 2023, no grant payments were made during the financial year 2023.

The payments made during the financial year amounted to £11,690.73. These consisted of mainly of travel expenses (c. 66%), conference admission fees (c. 12%), fees for outside counsel (c. 8%), IT expenses for website and email programmes (c. 5%), membership fees (c. 5%), with the remainder representing minor administrative expenses for items such a stationary and merchandising.

4.2. Statement of Assets and Liabilities as at 31st December 2023

	Dec 2022 £	Dec 2023 £
Fixed Assets		
Intangible Assets	-	-
Tangible Assets	-	-
Heritage Assets	-	-
Investments	-	-
Total Fixed Assets	-	-
Current Assets		
Stocks	-	-
Debtors	-	-
Investments	-	-
Cash at Bank	151,308.09	180,419.41
Total Current Assets	151,308.09	180,419.41
Liabilities		
Creditors: Amounts Falling Due Within One Year	-	-
Net Current Assets	151,308.09	180,419.41
Total Assets Less Current Liabilities	151,308.09	180,419.41
Creditors: Amounts Falling Due Within More Than One Year	-	-
Provision for Liabilities	-	-
Net Assets	151,308.09	180,419.41
Capital and Reserves		
Unrestricted Funds	151,308.09	180,419.41
Restricted Funds	-	-
Endowment Funds	-	-
Total Charity Funds	151,308.09	180,419.41

As of 31st December 2023, the only asset owned by the charity consisted of cash held on its two bank accounts. 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 2023 amounted to £180,419.41.



Harriet Holme,
Chair

Approved by the trustees on 24 May 2024

4.3. Cash-Flow Statement as at 31st December 2023

	Period ending 31st December 2022
Opening Cash as of 1st January 2023	151,308.09
Cash receipts during the year	40,802.05
Payments made during the year	-11,690.73
Net Movement of Funds	29,111.32
Closing Cash as of 31st December 2023	180,419.41

The charity's cash position evolved as a result of the inflow from donations exceeding the payments made during the financial year. As a result, the year-end cash position in its two bank accounts amounted to £180,419.41. This situation is expected to change during 2024 as the charity will begin to fund its first research project.

5. Notes to the Financial Statements for the period from 1st January 2023 to 31st December 2023

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

	1 st January 2023 £	Receipts £	Payments £	31 st December 2023 £
General Unrestricted Funds	151,308.09	40,802.05	-11,690.73	180,419.41
Designated Unrestricted Funds	0	0	0	0
Total Unrestricted Funds	151,308.09	40,802.05	-11,690.73	180,419.41
 Total Restricted Funds	 0	 0	 0	 0
 Total Endowment Funds	 0	 0	 0	 0
 Total	 151,308.09	 40,802.05	 -11,690.73	 180,419.41

As of 31st December 2023, 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 2023 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 2023 none of the trustees received any remuneration or benefits from an employment with the charity.

5.5. Related Party Transactions

In the period ending on 31st December 2023 the charity refunded one trustee expenses of £415.65. These were incurred in connection with IT expenses and travel to a conference. During the process of preparing these accounts it became apparent that within this total, £116 had been refunded in error and the trustee subsequently repaid the charity in January 2024.

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 2023, 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, with PCD Research contributing one third (£83,000) towards the project. The grant will be administered by PCD Research. In March 2023 the Scientific Advisory Panel decided to award the funds to a project team lead by Prof. Hart from University College, London (UCL). As of December 2023 the charity was continuing to negotiate the terms of the contract with UCL.

The grant contract between PCD Research and NATA was signed on 21 February 2024 and on 24 March 2024 the charity received the portion of the funding to be contributed by NATA (£167,000) into its accounts. PCD Research expects that the grant will be disbursed to UCL during the financial years 2024 and 2025.



Section A

Independent Examiner's Report

Report to the trustees/
members of

PCD Research

On accounts for the year
ended

Period ended 31 Dec 2023

Charity no
(if any)

1197528

Set out on pages

9-13 of the Annual Report and Accounts

(remember to include the page numbers of additional sheets)

I report to the trustees on my examination of the accounts of the above charity ("the Trust") for the year ended 31 Dec 2023.

Responsibilities and
basis of report

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:

9 Jun 2024

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