

Registered Charity Number: 1148034
Registered Company No: 7842342

CILIOPATHY ALLIANCE
(A company limited by guarantee)

FINANCIAL STATEMENTS
FOR THE PERIOD ENDED
31 MARCH 2013 (16 months)

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LEGAL AND ADMINISTRATIVE INFORMATION

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www.ciliopathyalliance.org

Registered Charity in England and Wales

Number 1148034 | 10 July 2012

Registered Company Limited by Guarantee

Number 7842342 | 10 November 2011

Directors and Trustees during period ended 31 March 2013:

Dr Thomas Kenny (resigned 10 May 2013)

Dr David Miller

Mr Michael Parker

Mr Stephen Thacker

Scientific Advisory Board

Prof Phil Beales (Chair)

Bankers

CAF Bank Ltd
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Accounts prepared by:

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Independent accounts examiner

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TRUSTEES' ANNUAL REPORT

The trustees of the Ciliopathy Alliance present their annual report and financial statements for the period ended 31 March 2013 (16 months), and confirm they comply with the requirements of the Companies Act 2006 and the Charities Act 1993, as amended by the Charities Act 2011. The report and financial statements will be laid before the members of the charity at the Annual General Meeting to be held on 18 September, 2013.

INTRODUCTION

The Ciliopathy Alliance is a new global alliance of patient support groups, researchers, doctors and allied health professionals representing patients and families living with and affected by diseases caused by defects in the function or structure of cilia.

Dysfunctional cilia are known to underlie a number of often chronically disabling and sometimes life-threatening genetic conditions. They affect multiple systems, causing blindness, deafness, chronic respiratory infections, kidney disease, heart disease, infertility, obesity and diabetes.

The last decade has seen spectacular and rapid advances in understanding the central role of cilia in developmental processes and given an insight into the molecular basis of numerous diseases that have emerged as 'ciliopathies'. Over 20 ciliopathies have been identified and more are suspected, with an estimated 1 in 1,000 people worldwide affected.

The ciliopathies currently represented by the Ciliopathy Alliance are:

- **Alström Syndrome:** ultra-rare, 700 known families worldwide (50-60 in UK) causing childhood blindness, hearing loss, heart, kidney and liver failure.
- **Bardet-Biedl Syndrome:** rare, 1 in 100,000 births, causing visual impairment, obesity, polydactyly, kidney abnormalities/renal failure, developmental delay, infertility.
- **Jeune Syndrome:** very rare, 1 in 200,000, causing skeletal malformations, lung/respiratory problems, renal cysts/renal failure.
- **Joubert Syndrome:** very rare, 1 in 100-250,000, causing ataxia (lack of muscle control), abnormal breathing pattern, sleep apnoea, abnormal eye and tongue movements.
- **Polycystic Kidney Diseases (PKD):** Autosomal Dominant PKD - 1 in 800 to 1,000 births, causing massive cystic kidneys and livers, kidney failure, brain aneurysms, cardiovascular disease; and the rare Autosomal Recessive PKD - 1 in 20-40,000 births, causing kidney failure and liver fibrosis, with stunted growth.
- **Primary Ciliary Dyskinesia:** 1 in 15,000, causing upper and lower respiratory tract infection, lungs, sinuses and ears.
- **Retinitis Pigmentosa:** 1 in 4,000, causing progressive sight loss.
- **Usher Syndrome:** 1 in 6-7,000 incidence, causing progressive hearing and sight loss, and balance problems.

GOVERNANCE, STRUCTURE AND MANAGEMENT

Governance

The Ciliopathy Alliance was established as a company limited by guarantee in 2011 and registered with the Charity Commission in 2012. Prior to becoming a registered charity, an alliance had been formed in 2010 of patient groups representing children and adults affected by ciliopathies, clinicians and scientists. These groups and individuals were the founding members of the charity. The charity is governed by the Memorandum and Articles of Association. The charity operates internationally.

The trustees of the charity, who are the directors of the limited company, are responsible for the governance of the charity. The charity had four founding trustees, three of whom were re-appointed at the first AGM on 10 May 2013. One new trustee, Professor Phil Beales, was appointed on 10 May 2013. Trustees are appointed by the charity. A minimum of two trustees must be present at each meeting for decisions to be made.

Trustees meet quarterly and communicate by email in between meetings. Members also meet quarterly at the same time as the trustees to allow input into the charity's strategy – considered crucial at this early stage in the charity's history and activities.

Trustees are provided with copies of relevant Charity Commission guidance and publications, including 'The Essential Trustee', 'Charities and Public Benefit' and 'It's your decision: charity trustees and decision making'.

All trustees are required to declare interests and may be required to withdraw from relevant proceedings during a board meeting. The trustees give their time freely and may claim reasonable out of pocket expenses.

Membership

Membership is open to any organisation or individual worldwide who shares the vision of the charity to 'improve the quality of life for people living with ciliopathies'.

Individuals and the nominated representatives of organisations can apply to the directors, who approve new members. The directors have established classes of membership with different rights and obligations.

A membership register is maintained by the secretariat. A list of members can be found on the Ciliopathy Alliance website. At 31 March 2013, the members were:

Patient Groups

Alström Syndrome UK
Laurence-Moon-Bardet-Biedl Society
Polycystic Kidney Disease Charity
Primary Ciliary Dyskinesia Family Support Group
RP Fighting Blindness

Individuals

Professor Phil Beales
Dr Claire Hogg
Dr Dan Jagger
Dr Tom Kenny
Dr David Miller
Dr Hannah Mitchison
Mr Stephen Thacker

Management

The trustees delegate the administration and secretariat functions of the charity to Tess Harris, an independent contractor, who is the CEO of the PKD Charity (a member of the Ciliopathy Alliance). The charity's accounts were prepared by Mr Phil Leeson, the finance director of Alström Syndrome UK (a member of the Ciliopathy Alliance) and were independently examined by Mr Chris Boid.

THE OBJECTS OF THE CHARITY AND ACTIVITIES FOR PUBLIC BENEFIT

The objects of the Ciliopathy Alliance are governed by the memorandum and articles of association and are as follows:

'To relieve sickness and promote and protect good health of children and adults living with ciliopathies, with a view to improving their conditions of life'.

To achieve these objects, the charity aims to:

- Promote, support and stimulate the sharing of knowledge and understanding of ciliopathies, nationally and transnationally.
- Encourage and facilitate communication between patients, health professionals, researchers and other organisations that support people with ciliopathies.
- Promote awareness of ciliopathies and the respective patient organisations.
- Promote, sponsor and/or participate in national and transnational laboratory, translational and clinical research into ciliopathies and related syndromes, with the aim of developing effective therapies and management of patients with ciliopathies.
- Invite and encourage patient involvement in research and clinical management of ciliopathies.
- Provide information that will benefit people with ciliopathies, in particular to those patients who do not have a condition/disease-specific support group and those in hard-to-reach geographical/ethnic communities.
- Provide information and educational materials for health professionals and similar with the aim of improving diagnosis, clinical management and social care.
- Communicate with/ lobby national and international governments and other relevant organisations to promote the interests of people with ciliopathies and encourage an integrated approach to their health and social care.
- Organise conferences, symposia, workshop and similar, and support the publication and/or dissemination of information and best practice about ciliopathies.
- Co-operate with other charities, alliances, voluntary organisations and statutory bodies to further the interests of people with ciliopathies.

All charitable activities are undertaken to further charitable purposes for public benefit. The trustees confirm they have referred to the guidance contained in the Charity Commission's general guidance on public benefit when reviewing the charity's aims and objectives, and in carrying out and planning current and future activities respectively.

ACTIVITIES AND ACHIEVEMENTS

CILIA2012 – the first international conference organised by the Ciliopathy Alliance

In May 2012, 270 researchers from 20 different countries participated in the CILIA2012 conference held at the UCL Institute of Child Health, in London. This was the first international scientific conference devoted to the role of cilia in development and disease. The proceedings provide an account of the scientific content of the meeting and contribute to describing the current state of cilia research during an exciting period for this rapidly expanding field. The proceedings of CILIA2012 are freely available on Cilia, an open-source journal from BioMed Central. <http://www.ciliajournal.com/content/1/S1/I1>

The conference sessions covered five main subject areas. These areas, with the keynote speakers who presented in each session, were: (1) Clinical and novel aspects of ciliopathies (Heymut Omran, University of Münster, Germany and Friedhelm Hildebrandt, University of Michigan, USA); (2) Structure and function of cilia (Brad Yoder, University of Alabama, USA and Greg Pazour, University of Massachusetts, USA); (3) Cilia and development (Kathryn Anderson, Sloan-Kettering Institute, USA and Jeremy Reiter, University of California San Francisco, USA); (4) Cilia and disease (Enza Maria Valente University of Messina, Italy, and Nicholas Katsanis, Duke University, USA); (5) Translational therapy and ciliotherapeutics (Peter Jackson, Genentech and Rachel Giles, University Medical Center Utrecht, Netherlands).

A concentrated schedule of talks and posters chosen from the submitted abstracts was assembled by the organising committee. This included significant unpublished data. Thirty-nine oral presentations were given, and 139 posters presented in total, covering many different diseases, numerous model organism systems, and including studies of almost all organs in the body. The plenary speaker was John Wallingford (University of Texas at Austin, USA), who spoke on 'Planar cell polarity, cilia and human disease'.

The scientific congress was preceded by a public engagement event that provided a unique opportunity for clinical and scientific researchers to meet with patients and relatives who suffer from ciliopathies, and other organisations working in their support. Joseph Gleeson (University of California San Diego, USA) gave the keynote talk at this event 'Translating gene discoveries for patient benefit'. Information stands were well attended by participants, organised by patient group members of the Ciliopathy Alliance. Those attending included EuroWABB, Polycystic Kidney Disease Charity, Primary Ciliary Dyskinesia Family Support Group, Laurence Moon Bardet Biedl Society, Joubert Syndrome in the UK, Kidney Research UK, Deafness Research UK and the Genetic Alliance/Rare Disease UK.

Information and awareness

The Ciliopathy Alliance website www.ciliopathyalliance.org was launched in October 2011 and attracted over 58,000 unique visitors in the period to 31 March 2013. The site contains information on the structure and function of cilia (the most popular pages on the site) and links to the patient support groups.

Planning the first Ciliopathy Alliance Family Conference

In February 2012, the charity was awarded a £25,000 grant from Jeans for Genes (Genetic Disorders UK) to organise a family conference in spring 2013, with the aim to:

- Update families and clinicians on latest research and best clinical practices.
- Enable families to give feedback on patient experiences as input to support groups and specialised services commissioners.
- Bring families/patients together with clinicians, researchers and commissioners.
- Provide a forum to pool knowledge and expertise.
- Develop shared strategies for improving patient care.
- Provide help to patients without support groups to form a group.
- Input into the charity's future strategy.

At the date of this document, the trustees are pleased to report that the Family Conference was attended by nearly 100 families, clinicians, researchers and allied health professionals. The proceedings of the Conference, including the plenary talks and a graphical record of the discussions, can be found on the Ciliopathy Alliance website <http://www.ciliopathyalliance.org/events/past-events/117-ciliopathy-alliance-family-conference-2013.html>

FUTURE PLANS

The long-term sustainability of the Ciliopathy Alliance is a primary focus and during the year to 31 March 2014, the charity will actively seek new sources of funding. At a strategy meeting in January 2013, trustees and members together identified key themes of: Patient Engagement, Knowledge Sharing, Policy Influence and Promotion of Therapeutic Research and Development. Within each theme, during the year, the directors (supported by members) intend to elaborate on the major issues and identify gaps/opportunities for the charity to develop activities, within its resource limitations.

During the year, the charity will work on the development of a 'Route Map' for families in the UK, using a toolkit provided by Genetic Alliance UK. This work builds on the output from the Family Conference held in spring 2013.

During the year, the charity will work with academic colleagues in the planning of CILIA2014, the second international conference on cilia, which will be held in Paris in November 2014. The conference will include a patient/family engagement event.

The charity will also consider a research strategy and consider ways to promote and increase membership.

FINANCIAL REVIEW

Performance

During the period ended 31 March 2013 (16 months), the charity's income was £83,982 (previous: zero).

The charity incurred expenses of £73,018 resulting in a surplus of £10,964.

The primary source of income in the year was from delegate fees and sponsorship for the CILIA2012 Conference.

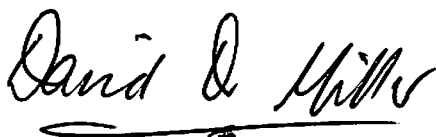
Risk Management

The directors have overall responsibility for ensuring that the Ciliopathy Alliance is managing risk in a professional, responsible and constructive manner. This has involved identifying risks the charity may face, assessing potential impact and minimising them.

Reserves

The directors considered that the charity had sufficient reserves at the year-end to fund its proposed activities during 2013-14.

Approved by the Directors and signed on their behalf by



David D Miller
Chair

18th Sep 2013

FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 MARCH 2013

Statement of Financial Activities	Unrestricted Funds	Restricted Funds	Total 2013	Total 2012	See Note
	£	£	£	£	
Incoming Resources					
Incoming Resources From Generated Funds: -					
Voluntary Income	1,250	0	1,250	0	2
Activities For Generating Funds	0	0	0	0	
Investment Income	0	0	0	0	
Incoming Resources From Charitable Activities	82,699	0	82,699	0	3
Other Incoming Resources	33	0	33	0	4
Total Incoming Resources	83,982	0	83,982	0	
Resources Expended					
Costs of Generating Funds					
Costs of Generating Voluntary Income	0	0	0	0	2
Fundraising Trading - Costs of Goods Sold & Other Costs	0	0	0	0	
Investment Management Costs	0	0	0	0	
Charitable Activities	66,151	0	66,151	0	3
Governance Costs	6,673	0	6,673	0	4
Other Resources Expended	194	0	194	0	5
Total Resources Expended	73,018	0	73,018	0	
Net Incoming/Outgoing (-) Resources Before Transfers	10,964	0	10,964	0	
Transfers					
Gross Transfers Between Funds	0	0	0	0	
Net Incoming Resources Before Other Recognised Gains & Losses	10,964	0	10,964	0	
Other Recognised Gains / Losses					
Gains on Revaluation of Assets for Charity's Own Use	0	0	0	0	
Gains / Losses on Investment Assets	0	0	0	0	
Actuarial Gains / Losses on Defined Benefit Pension Schemes	0	0	0	0	
Net Movement in Funds	10,964	0	10,964	0	
Reconciliation of Funds					
Total Funds Brought Forward	0	0	0	0	
Total Funds Carried Forward	10,964	0	10,964	0	

Balance Sheet		2013	2012	See Note
		£	£	
Fixed Assets				
	Total Fixed Assets	0	0	8
Current Assets				
	Stocks & Work-in-Progress	0	0	
	Debtors	0	0	
	Investments	0	0	
	Cash at Bank & In Hand	35,964	0	9
	Total Current Assets	35,964	0	
Liabilities				
	Creditors - Amounts Falling Due Within 1 Year	25,000	0	10
	Net Current Assets or Liabilities	10,964	0	
	Total Assets less Current Liabilities	10,964	0	
	Creditors - Amounts Falling Due After 1 Year	0	0	
	Provisions for Liabilities & Charges	0	0	
	Net Assets or Liabilities	10,964	0	
The Funds of the Charity				
	Restricted Funds	0	0	
	Unrestricted Funds	10,964	0	11
		10,964	0	

NOTES TO THE STATEMENT OF FINANCIAL ACTIVITIES & BALANCE SHEET

1 Accounting Policies - The principal accounting policies are summarised below. The accounting policies have been applied consistently throughout the year and in the preceding year.

(a) Basis of Accounting

The financial statements have been prepared under the historical cost convention and in accordance with the Companies Act 1985 and the Statement of Recommended Practice : Accounting and Reporting by Charities issued in March 2005

(b) Fund Accounting

- Unrestricted funds are available for use at the discretion of the trustees in furtherance of the general objectives of the charity.
- Restricted Funds are subjected to restrictions on their expenditure imposed by the donor.

(c) Incoming Resources

All incoming resources are included in the statement of financial activities when the charity is entitled to, and

virtually certain to receive, the income and the amount can be quantified with reasonable accuracy. The following policies are applied to particular categories of income:

- Voluntary income is received by way of grants, donations and gifts and is included in full in the Statement of Financial Activities when receivable. Grants, where entitlement is not conditional on the delivery of a specific performance by the charity, are recognised when the charity becomes unconditionally entitled.
- Investment income is included when it is earned.
- Incoming resources from grants, where related to performance and specific deliverables, are accounted for as the charity earns the right to consideration by its performance.

(d) Resources Expended

Expenditure is recognised on an accrual basis as a liability is incurred. Expenditure includes any VAT which cannot be fully recovered, and is reported as part of the expenditure to which it relates:

- Costs of generating funds comprise the costs associated with attracting voluntary income and the cost of investing and trading for fundraising purposes.
- Charitable expenditure comprises those costs incurred by the charity in the delivery of its activities and services for its beneficiaries. It includes both costs that can be allocated directly to such activities and those costs of an indirect nature necessary to support them.
- Governance costs include those costs associated with meeting the constitutional and statutory requirements of the charity and include the audit fees and costs linked to the strategic management of the charity.
- The nature of the charity is such that all costs are allocated directly to the expenditure categories of the SoFA without need for apportionment.

(e) Fixed Assets

The nature of the charity is such that it holds no fixed assets.

- 2 Voluntary Income - relates to monies kindly donated by individuals and charitable organisations either for specific purposes (restricted funds) or for the general benefit of the charity (unrestricted funds). No costs were expended.
- 3 Charitable Activities - the main purpose of the charity is to promote, support and stimulate the sharing of knowledge and understanding of ciliopathies, nationally and transnationally. To achieve this the charity organises conferences, workshops etc which gave rise to all of the income and expenditure stated.
- 4 Governance Costs - costs relating to the general running of the charity as opposed to the direct management function. Includes directors' costs, preparation of statutory accounts etc.
- 5 Other Resources Expended - relate specifically to finance costs.
- 6 Trustee Remuneration & Third Party Transactions – no Trustee received any remuneration during the year or the previous year. No Trustee or other person related to the charity had any personal interest in any contract entered into by the charity during the year or the previous year.
- 7 Taxation - the charity is exempt from tax on income and gains falling within section 505 of the Taxes Act 1988 or s256 of the Taxation of Chargeable Gains Act 1992 to the extent that these are applied to its charitable objects. No tax charges have arisen in the charity.
- 8 Fixed Assets - the charity does not own any fixed assets.
- 9 Cash - Monies held in various bank accounts available for investment and to meet day-to-day running expenses.
- 10 Creditors - relates to income received in advance of next year's activities.
- 11 The fund balances held by the charity at 31 March 2013 were all unrestricted and available for use at the discretion of the trustees in furtherance of the general objectives of the charity.

INDEPENDENT EXAMINER'S REPORT TO THE TRUSTEES OF CILIOPATHY ALLIANCE

I report on the accounts of the charity for the year ended 31st March 2013, which are set out above.

Respective Responsibilities of Trustees and Examiner

The trustees (who are also the directors of the company for the purposes of company law) are responsible for the preparation of the accounts. In accordance with section 145(1) of the Charities Act 2011, the trustees have elected to subject the accounts to independent examination and, as a member of the Chartered Institute of Public Finance and Accountancy, I am qualified to undertake that examination.

Having satisfied myself that the charity is not subject to audit under company law and is eligible for independent examination, it is my responsibility to:

- examine the accounts under section 145 of the 2011 Act;
- to follow the procedures laid down in the general Directions given by the Charity Commission under section 145(5)(b) of the 2011 Act; and
- to state whether particular matters have come to my attention.

Basis of Independent Examiner's Report

My examination was carried out in accordance with the general Directions given by the Charity Commission. An examination includes a review of the accounting records kept by the charity and a comparison of the accounts presented with those records. It also includes consideration of any unusual items or disclosures in the accounts, and seeking explanations from you as trustees concerning any such matters. The procedures undertaken do not provide all the evidence that would be required in an audit and consequently no opinion is given as to whether the accounts present a 'true and fair view' and the report is limited to those matters set out in the statement below.

Independent Examiner's Statement

In connection with my examination, no matter has come to my attention:

(1) which gives me reasonable cause to believe that, in any material respect, the requirements:

- to keep accounting records in accordance with section 386 of the Companies Act 2006; and
- to prepare accounts which accord with the accounting records, comply with the accounting requirements of section 396 of the Companies Act 2006 and with the methods and principles of the Statement of Recommended Practice: Accounting and Reporting by Charities

have not been met; or

(2) to which, in my opinion, attention should be drawn in order to enable a proper understanding of the accounts to be reached.

Chris Boid

Chartered Institute of Public Finance & Accountancy

**32 Alma Row
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Rotherham
S60 4HT**

15 August 2013