REGISTERED COMPANY NUMBER: 07842342 (England and Wales)
REGISTERED CHARITY NUMBER: 1148034

REPORT OF THE TRUSTEES AND UNAUDITED FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 MARCH 2020 FOR

CILIOPATHY ALLIANCE
(A COMPANY LIMITED BY GUARANTEE)

CONTENTS

Trustees

Professor Philip Beales (Professor of Medical Genetics)
Mrs Claire Glen (University Administrator) (Retired 11 December 2019)
Dr Elizabeth Forsythe (Clinician)
Dr Toby Hurd (Researcher)
Dr David Miller (Chair) (Independent Consultant)
Professor Hannah Mitchison (Researcher)
Mr Michael Parker (Retired Actuary)
Mr Stephen Thacker (Charity Volunteer)

Scientific Advisory Board

Chaired by Professor Philip Beales

Secretariat
Delegated to Miss Tess Harris

Registered Office

91 Royal College Street
London
NW1 0SE

Telephone number: 020 7387 0543
Email address: info@ciliopathyalliance.org
Web address: www.ciliopathyalliance.org

Registered Company Number
07842342 (England and Wales) (Incorporated 10 November 2011)

Registered Charity Number
1148034 (registered 10 July 2012)

Bankers
CAF Bank Limited
25 Kings Hill Avenue
West Malling
Kent
ME14 4JQ

The trustees, who are also directors of the charity for the purposes of the Companies Act 2006, present their report with the financial statements of the charity for the year ended 31 March 2020. The trustees have adopted the provisions of Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2015).

INTRODUCTION

The Ciliopathy Alliance is a 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.

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

Individual disorders caused by malfunctioning cilia are rare, but more than 100 diseases have been identified - known collectively as 'ciliopathies' - and they affect as many as one in 500 - 1,000 people.

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 prevalence, causing visual impairment, obesity, polydactyly, kidney abnormalities/renal failure, developmental delay, infertility.
- Jeune Syndrome: very rare, 1 in 200,000 prevalence, causing skeletal malformations, lung/respiratory problems, renal cysts/renal failure.
- Joubert Syndrome: very rare, 1 in 100,000 - 250,000 prevalence, 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 1,000 - 4,000 prevalence, causing massive cystic kidneys and livers, kidney failure, brain aneurysms, cardiovascular disease; and the rare Autosomal Recessive PKD - 1 in 20,000 - 40,000 prevalence, causing kidney failure and liver fibrosis, with stunted growth.
- Primary Ciliary Dyskinesia: 1 in 15,000 prevalence, causing upper and lower respiratory tract infection, and disorders of the lungs, sinuses and ears.
- Retinitis Pigmentosa: 1 in 4,000 prevalence, causing progressive sight loss.
- Usher Syndrome: 1 in 6,000 - 7,000 prevalence, causing progressive hearing and sight loss, and balance problems.

OBJECTIVES AND ACTIVITIES

Objectives and aims

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.

SIGNIFICANT ACTIVITIES

Covid-19

The Covid-19 epidemic broke out shortly before the end of this reporting period. The Trustees responded by cancelling face to face meetings. At the time of preparing this report, there are still many restrictions which hinder the normal operation of the charity.

Information and awareness

Following the great success of Cilia 2018 in Copenhagen, the organising baton passed to Prof Bernhard Schermer, University Hospital Cologne, as leader of the conference organising committee for Cilia 2020, to be held in that city in October 2020. Represented by Miss Harris, the Ciliopathy Alliance continues its standing membership on the committee, advocating strongly for the maximum inclusion of patient group participation, especially in seeking avenues to facilitate attendance. Unfortunately, uncertainties around Covid-19 restrictions precipitated a decision to postpone the conference by one year and plans continue to be put in place for Cilia 2021. www.cilia2021.de

During the year, Professor Beales continued to lead for the charity on liaison with NHS England (NHSE) on plans to commission a highly specialised service for ciliopathy patients. Throughout the whole process, despite there having been slippages in the approval process timeline, it had been hoped that the service would still be commissioned by NHSE in 2019. However, NHSE eventually indicated that they would not go ahead with commissioning the service. This appears to reflect changing processes and priorities within NHSE during the protracted period the application was in the pipeline, and a background of increasingly limited resources. An alternative commissioning model, suggested by an NHSE representative, could be the development of a Rare Disease Collaborative Network (RDCN). In addition, Miss Harris attended various meetings on rare diseases organised by Genetic Alliance/RDUK, inter alia.

January 2020 saw the launch of an EU Horizon 2020 funded consortium – the European Training Network for Studying Ciliary Signalling in development and disease, 'SCilS'. SCilS combines the expertise of eight academic, four industrial, and two non-profit sector partners from across Europe and it aims to train a new generation of multidisciplinary researchers and entrepreneurs specialising in primary cilia and ciliopathies. The Ciliopathy Alliance is one of the non-profit partners and contributes to patient engagement training.

Multiple letters of support have been provided to researchers applying for research funding related to cilia and ciliopathies.

The Ciliopathy Alliance website www.ciliopathyalliance.org attracted 30,000 visitors during the year from a wide range of

countries. 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. The charity maintains an active Twitter account with 700 followers, primarily scientists and clinicians interested in cilia and ciliopathies.

Trustees

The trustees recognise their obligations to maintain a dynamic board of trustees and as part of reviewing and refining its management of trusteeships, the Ciliopathy Alliance has initiated a rolling programme of new trustee appointments in preparation for eventual retirement of longer-standing trustees.

Public benefit

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.

FINANCIAL REVIEW

Performance

During the year ended 31 March 2020, the charity's income was £13 (2019: £7).

The charity incurred expenses of £735 (2019: £2,326) resulting in a deficit of £722 (2019: deficit £2,319).

Full details of the charity's performance are set out in page 8 of the statement of financial activities to the financial statements.

Reserves policy

The directors consider that, when combined with expected income, the charity had sufficient reserves at the year-end to fund its routine activities during 2020-21. If, however, activities that require a more substantial funding base are to be undertaken, the directors accept that more vigorous fundraising activities will need to be pursued.

FUTURE PLANS

The charity aims to continue the development of objectives that support its aims, and the focus will then be on fundraising to implement the plan and to sustain the charity.

The website will be maintained and kept up to date.

The Ciliopathy Alliance is a member of the steering committee of the fifth international ciliopathies conference, Cilia 2021, which will take place in Cologne in the autumn of 2021.

STRUCTURE, GOVERNANCE AND MANAGEMENT

Governing document

The Ciliopathy Alliance was established as a company limited by guarantee in 2011 and registered with the Charity Commission in 2012. The charity is governed by its Memorandum and Articles of Association.

Organisation structure and governance

The trustees of the charity, who are the directors of the limited company, are responsible for the governance of the charity. The charity has seven trustees. Trustees are appointed by the charity based on their skills and expertise. A minimum of two trustees must be present at each meeting for decisions to be made.

Trustees meet approximately quarterly and communicate by email in between meetings. Members' meetings are held regularly to allow sharing of knowledge and input into the charity's strategy.

Trustees are provided with copies of relevant Charity Commission guidance and publications.

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 but may claim reasonable out of pocket expenses.

Management

The trustees delegate the administration and secretariat functions of the charity to Ms Tess Harris, a volunteer, who is the CEO of the PKD Charity (a member of the Ciliopathy Alliance). The charity's accounts were prepared by Ms Susan Hartley (volunteer bookkeeper). The charity was exempt from the requirement to have an Independent Examination.

Risk management

The trustees have a duty to identify and review the risks to which the charity is exposed and to ensure appropriate controls are in place to provide reasonable assurance against fraud and error.

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 for membership, which is subject to the directors' approval. The directors have established classes of membership with different rights and obligations.

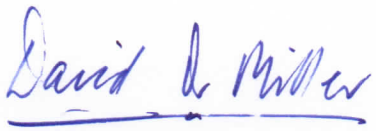
A membership register is maintained by the secretariat. At 31 March 2020, the members were:

Patient Groups
Alström Syndrome (UK)
Bardet-Biedl Syndrome (UK)
Jeune Syndrome (UK)
Joubert Syndrome (UK)
Polycystic Kidney Disease Charity (UK)
Primary Ciliary Dyskinesia Family Support Group (UK)
RP Fighting Blindness (UK)
Sense (UK)
Dyskinesia (Belgium)
Sensenbrenner Syndrome patient group (Poland)
Usher Syndrome Coalition (international)

Individuals
Professor Philip Beales
Dr Sonia Christou-Savina
Dr Megan Davey
Dr Elizabeth Forsythe
Mrs Claire Glen
Dr Jay Gopalakrishnan
Dr Claire Hogg
Dr Toby Hurd
Dr Dan Jagger
Dr Swadhin Jana
Dr David Miller
Prof Hannah Mitchison
Dr Brian Piasecki
Dr Ursula Rodgers
Dr Miriam Schmidts
Mr Stephen Thacker
Dr Joanna Walczak-Sltulpa
Dr Gabrielle Wheway

Organisations
Cincinnati Children's Hospital
National Institute of Sensory Organs
Poznan University of Medical Sciences

Approved by order of the board of trustees on 7 December 2020 and signed on its behalf by:



Dr D D Miller - Trustee

	Unrestricted fund £	Restricted fund £	2020 Total funds £	2019 Total funds £
INCOME AND ENDOWMENTS FROM				
Donations and legacies	13	-	13	7
Total	13	-	13	7
EXPENDITURE ON				
Charitable activities				
Conferences and workshops	-	-	-	1,651
Membership subscription	22		22	
Bank charges	60		60	60
Website costs	246		246	319
Governance costs				
Trustee expenses	394		394	284
Accountancy fees	-		-	840
Companies House	13		13	13
Total resources expended	735	-	735	2,326
NET INCOME	-722	-	-722	-2,319
RECONCILIATION OF FUNDS				
Total funds brought forward	7,901	-	7,901	10,220
TOTAL FUNDS CARRIED FORWARD	7,179	-	7,179	7,901

CONTINUING OPERATIONS
All income and expenditure has arisen from continuing activities.

BALANCE SHEET
AT 31 MARCH 2020

	Unrestricted fund £	Restricted fund £	2020 Total funds £	2019 Total funds £
CURRENT ASSETS				
Cash at bank	7,179	-	7,179	7,901
CREDITORS				
Amounts falling due within one year	-	-	-	-
NET CURRENT ASSETS	7,179	-	7,179	7,901
TOTAL ASSETS LESS CURRENT LIABILITIES		-		7,901
NET ASSETS	7,179	-	7,179	7,901
FUNDS				
Unrestricted funds			7,179	7,901
Restricted funds			-	-
TOTAL FUNDS			7,179	7,901

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 March 2020.

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2020 in accordance with Section 476 of the Companies Act 2006.

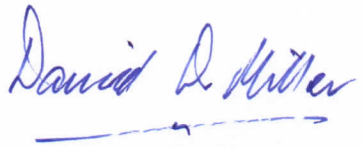
The trustees acknowledge their responsibilities for

(a) ensuring that the charitable company keeps accounting records that comply with Sections 386 and 387 of the Companies Act 2006 and

(b) preparing financial statements which give a true and fair view of the state of affairs of the charitable company as at the end of each financial year and of its surplus or deficit for each financial year in accordance with the requirements of Sections 394 and 395 and which otherwise comply with the requirements of the Companies Act 2006 relating to financial statements, so far as applicable to the charitable company.

These financial statements have been prepared in accordance with the special provisions of Part 15 of the Companies Act 2006 relating to charitable small companies.

The financial statements were approved by the Board of Trustees on 7 December 2020 and were signed on its behalf by:



Dr D D Miller -Trustee