

Charity number: 1182818

Company number: CE017079

(England and Wales)

ACRODYSOSTOSIS SUPPORT & RESEARCH

Report of the Trustees and Unaudited Financial Statements

For the year ended 31 December 2023

ACRODYSOSTOSIS SUPPORT & RESEARCH
Contents Page
For the year ended 31 December 2023

Report of the Trustees	1 to 4
Independent Examiner's Report to the Trustees	5
Statement of Financial Activities	6
Statement of Financial Position	7
Notes to the Financial Statements	8 to 12
Detailed Statement of Financial Activities	13

ACRODYSOSTOSIS SUPPORT & RESEARCH

Report of the Trustees

For the year ended 31 December 2023

The Trustees, who are also directors for the purposes of company law, have pleasure in presenting their report and the financial statements for the charitable company for the year ended 31 December 2023. 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 the Republic of Ireland (FRS 102) (effective 1 January 2015).

Chair's report

Statement of Trustees' responsibilities

The Trustees are responsible for preparing the Trustees' report and the accounts in accordance with Charities Act 2011 and United Kingdom Generally Accepted Accounting Practice.

The Charities Act 2011 requires the Trustees to prepare accounts for each financial year which give a true and fair view of its financial activities during the year and of its financial position at the end of the year. In presenting those accounts, they are required to:

- select suitable accounting policies and then apply them consistently;
- observe the methods and principles in the Charities SORP FRS 102;
- make judgements and estimates that are reasonable and prudent;
- follow applicable UK accounting standards and the Charities SORP FRS 102, disclosing and explaining any departures in the accounts; and
- prepare the accounts on the going concern basis unless it is inappropriate to do so.

The Trustees are responsible for maintaining proper accounting records which disclose with reasonable accuracy at any time the financial position of the Charity and to enable them to ensure that the accounts comply with the Charities Act 2011 and with the requirements of the Statement of Recommended Practice 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 (FRS102) (effective 1 January 2015) - Charities SORP FRS102), the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS102). 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 besides the above responsibilities.

This report was approved by the trustees on 17th October 2024

OBJECTIVES AND ACTIVITIES

Charitable objectives:

To relieve the needs of those affected by Acrodysostosis by promoting greater understanding of the causes, symptoms, and treatment of Acrodysostosis in all its forms including:

- promoting research and sharing and disseminating of results of such research for the benefit of the general public; and
- raising public awareness of the symptoms, needs and related medical conditions of those living with Acrodysostosis.

Charitable activities:

The Trustees ensure that sufficient attention is paid to fundraising activities, monitoring grant funding opportunities and manage the charity's assets to ensure its sustainability and ability to carry out its charitable objectives. The charity relies heavily on its volunteers as it has no paid members of staff.

Objectives and aims

In exercising their duty, the Trustees confirm that they have considered the Charity Commission's guidance on public benefit, including the guidance 'public benefit: running a charity (PB2)'.

The charity's objectives are to offer support and information to families affected by Acrodysostosis. Acrodysostosis Support & Research liaises closely with researchers at UCL Great Ormond St Hospital (UCL GOSH) with regards to the ongoing pioneering research into Acrodysostosis. Additionally, the charity is actively engaged in collaborative efforts with researchers from the University of Manchester, University of Glasgow and Keele University and also works constructively and in partnership with other agencies and organisations to continually improve support for families affected by Acrodysostosis - to change lives and make a difference to those living with the condition.

ACRODYSOSTOSIS SUPPORT & RESEARCH

Report of the Trustees Continued For the year ended 31 December 2023

ACHIEVEMENTS AND PERFORMANCE

Key activities and achievements throughout the year include:

Peer Support

- One of the main aims of the charity is to bring together families and the Acrodysostosis community to share experiences and provide peer support through the Acrodysostosis network. The charity provides this support to their families via their ambassadors who are always available to listen and provide emotional and practical help to their Acrodysostosis Families.
- Acrodysostosis have continued to provide a range of information offering guidance and support for Acrodysostosis families.

General Support

Acrodysostosis families have found comfort from hearing and sharing experiences via the Acrodysostosis private Support Facebook group as well as various zoom meetings which any of the Acrodysostosis community could attend and get the opportunity to meet virtually.

Acrodysostosis Support & Research have continued to provide a range of information offering guidance and support for Acrodysostosis families including:

Medical Advisory Board (MAB):

The MAB was formed to guide the trustees in scientific matters, including research efforts and direction, and consists of renowned experts in the field. The MAB continues to meet regularly to facilitate the expanding body of knowledge around Acrodysostosis so families can be better supported - the trustees are very grateful to the MAB members for their dedication and commitment to helping Acrodysostosis families. The MAB along with Trustees and Ambassadors are continuing to take the lead in identifying research opportunities. And through a roundtable meeting which was sponsored by Costello medical the charity was able to bring together all the researchers, MAB, Trustee and Ambassadors to discuss the future landscape for research into Acrodysostosis with the aim of finding treatments.

Communications:

- Communications with those within the Acrodysostosis community and the general public through the Acrodysostosis website, and via social media.
- On Facebook a public page for the general public is used as their primary form of outbound communication. The charity also has a closed support group for families affected by the condition.
- Acrodysostosis is also active on LinkedIn, Twitter and Instagram pages.

Research:

- Acrodysostosis Support & Research is actively collaborating with its Medical Advisory Board (MAB) and the Acrodysostosis Community to introduce a global Acrodysostosis patient registry. Simultaneously, the charity has been diligently gathering data through the first-ever community survey in Acrodysostosis.
- In February 2023 the article "Growth patterns and outcomes of growth hormone therapy in patients with acrodysostosis" by Ertl, Diana-Alexandra was published online in Journal of Endocrinological Investigation. Along with Ertl, Diana-Alexandra, this was a collaboration between Acrodysostosis Support & Research and their community. The Chair and on one of the Ambassadors have been included as co-authors. This is part of the Springer Nature Content Sharing Initiative, here is the link to view the paper: <https://rdcu.be/c479M>
- With the support of Acrodysostosis Support and Research Dr Melissa Bowerman and Prof George Baille were awarded a grant of £1,036,199.30 for 3 years from the MRC here is summary: They have recently generated a new mouse model for ACRDYS 2 that presents similar features to the human condition such as a small size, reduced weight and abnormal brain function. They propose to firstly further characterise this novel mouse model, whereby the animals will be observed to record the range of symptoms that occur in ACRDYS 2 patients. They will also evaluate the effect of PDE4D blocking drugs (existing and newly developed) on the symptoms and other regulatory molecules in this mouse model. This novel mouse model will be an invaluable tool for scientists, doctors and patients to help better understand the different aspects of the disease and importantly, develop new treatments that can prevent and/or slow down the symptoms in ACRDYS 2 and other conditions where activity of PDE4D is abnormally increased.
- In 2023, with the invaluable support of Acrodysostosis Support & Research, Professor Sue Kimber continues her 5-year project awarded by the Medical Research Council (MRC) and the National Institute for Health and Care Research (NIHR). This represents a significant milestone in the ongoing efforts to advance the understanding of rare conditions including Acrodysostosis. Here is a summary: In patients with Acrodysostosis, genetic mutations disrupt the response of growth plate cartilage to hormones. A research team has developed a method to cultivate growth plate-like cartilage from stem cells and manipulate genes. Their project aims to replicate these genetic mutations in stem cells, generating defective cartilage for comparison with normal cartilage. By analysing the

ACRODYSOSTOSIS SUPPORT & RESEARCH

Report of the Trustees Continued For the year ended 31 December 2023

RNA molecules responsible for protein coding, they hope to pinpoint the molecular-level issues in cartilage formation. This research is expected to identify key molecules causing the disease, potentially paving the way for future drug treatments.

- In 2023, Acrodysostosis Support & Research continues to collaborate with Great Ormond Street Hospital Children's Charity (GOSHCC) & Sparks, as part of their national funding call for 2020/21. This initiative is dedicated to advancing the understanding and treatment of rare diseases in children. The successful application was publicly announced in April 2021, and the grant was awarded to Professor Patrizia Ferretti at University College London (UCL). The primary objective of this partnership is to develop human models of acrodysostosis, with the goal of identifying specific druggable targets tailored to each patient's disease, aiming to normalize development in affected children and enhance their overall quality of life.

As a significant contributor to this venture, Acrodysostosis Support & Research did provide 25% of the project's total funding, equivalent to £62,500 in 2021. The organisation is delighted to confirm that this three-year research project commenced in the autumn of 2021. Regular communication between the charity and the research team ensures that the community stays updated on progress. Additionally, the charity remains open to exploring opportunities for future collaborative projects.

Raising Awareness:

- In March 2023 The charity attended, the Advanced Therapies Congress which is Europe's largest cell and gene therapy conference and exhibition in London. It's attended by 2,000 delegates, 100 exhibitors, 200 innovative young start-ups and 300 speakers from across the entire value chain of cell and gene therapy development; including forward-thinking pharma companies, researchers, clinicians, academics, HTAs, payers and regulators.

- In June 2023 The chair of the charity, Nina Knight, as a programme committee member for the EURORDIS Open Academy School went to Barcelona to attend and complete the Open Academy School on Scientific Innovation and Translational Research' as well as moderate one of the sessions. The school is attended by 100's of rare disease advocates as well as thought leaders including, researchers, clinicians, academics, HTAs, and regulators.

- In September 2023 Nina Knight the chair of the charity became an ePAG@ERN-ITHACA (patient advisory board member at the European reference network for rare malformation syndromes and rare intellectual and neurodevelopmental disorders). The role of ePAG is to represent the voice and interests of their patient community and act as the bridge between this community and the ERN. ePAG have an official permanent mandate to represent ePAG member organisations. They liaise with these organisations to ensure true and equitable representation of the patient voice by participating in the Board and sub-clinical committees of their respective ERN.

In October 2023 The charity attended The World Orphan Drug Congress Europe which is the largest and most established orphan drug & rare disease event worldwide. Where the charity had the opportunity to connect with more than 2000 attendees, 250 leading speakers, and 130 exhibitors, as they bring together experts from the start-to-finish of orphan drugs, from regulation and policy to global pricing and gene therapy.

- In October 2023 The Charity attended the 4th Annual MarketsandMarkets Orphan Drugs and Rare Diseases Conference in London, which aims to provide a valuable and broad platform to discuss recent advances in the development of life-saving therapies, critical issues, and strategies to enhance orphan drug development in near future. The conference brought together scientists, researchers, entrepreneurs, academicians, medical officials, and technologists across the globe to discuss the newest findings in the areas of rare diseases and orphan drugs, helping to shape present and upcoming challenges in drug analysis. It gave the charity the opportunity to interact with industry peers and participate in a scientific conference while staying informed about the most recent developments in the study of rare diseases.

- In November 2023 The charity attended the Rare Beacon <https://www.rarebeacon.org/> London Rare Disease Showcase' which aims to shed light on the latest innovations and advancements occurring in the field of rare diseases, with special emphasis on the vibrant rare disease landscape in the UK's capital.

Fundraising:

- The charity received a generous donation of £200 from a parent of a child with Acrodysostosis.

- The charity received £4755.27 in gift aid.

- Furthermore, in addition to the financial contributions from parents, patients, and friends of the charity, a substantial number of volunteer hours have been dedicated to Acrodysostosis Support & Research. These individuals have passionately supported families and remain steadfast in their commitment to advancing the scientific understanding of this disease. Their collective efforts are focused on expediting the development of viable treatments and, ideally, a cure in the future.

ACRODYSOSTOSIS SUPPORT & RESEARCH
Report of the Trustees Continued
For the year ended 31 December 2023

FINANCIAL REVIEW

Financial review and reserves policy

The charity Trustees continually monitor cash reserves to ensure its financial stability.

Acrodysostosis Support & Research is committed to building a reserves policy to ensure there is enough funding to cover one year's operating costs.

The charity has no outstanding financial obligations nor outstanding debts. The charity's principal source of funds comes from the general public, either through donations or at one-off fundraising events.

Going concern

The Trustees have assessed the charities finances and can confirm there is no reason why the chairty will not continue to operate for at least 12 months passed the date of approval of these accounts.

REFERENCE AND ADMINISTRATIVE INFORMATION

Name of Charity	ACRODYSOSTOSIS SUPPORT & RESEARCH
Charity registration number	1182818
Company registration number	CE017079
Principal address	Bath House, Cudworth Lane Newdigate Dorking Surrey SE15 5AF

Trustees

The trustees and officers serving during the year and since the year end were as follows:

Jan Sara Hine
Sarah Louise Denney
Nina Samantha Knight

Independent examiners

Beach Accountants Limited
Chartered Certified Accountants
10 Blue Sky Way
Monkton Business Park South
South Tyneside
NE31 2EQ

Approved by the Board of Trustees and signed on its behalf by

DocuSigned by:

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Nina Samantha Knight

17 October 2024

ACRODYSOSTOSIS SUPPORT & RESEARCH
Independent Examiners Report to the Trustees
For the year ended 31 December 2023

I report to the trustees on my examination of the accounts of the charitable company for the year ended 31 December 2023.

Responsibilities and basis of report

As the charity Trustees, who are also directors for the purposes of company law, are responsible for the preparation of the accounts in accordance with the requirements of the Companies Act 2006 ('the 2006 Act').


Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts carried out under section 145 of the Charities Act 2011 ('the 2011 Act'). In carrying out my examination I have followed the Directions given by the Charity Commission under section 145(5)(b) of the 2011 Act.

Independent examiners statement

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

1. accounting records were not kept in respect of the Company as required by section 386 of the 2006 Act; or
2. the accounts do not accord with those records; or
3. the accounts do not comply with the requirements of section 396 of the 2006 Act other than any requirement that the accounts give a 'true and fair view' which is not a matter considered as part of an independent examination; or
4. the accounts have not been prepared in accordance with the methods and principles of the Statement of Recommended Practice for accounting and reporting by charities applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102).

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

DocuSigned by:

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Beach Accountants Limited
Chartered Certified Accountants
10 Blue Sky Way
Monkton Business Park South
South Tyneside
NE31 2EQ

17 October 2024

ACRODYSOSTOSIS SUPPORT & RESEARCH
Statement of Financial Activities (including Income and Expenditure Account)
For the year ended 31 December 2023

	Notes	Unrestricted funds £	Restricted funds £	2023 £	2022 £
Income and endowments from:					
Donations and legacies	2	5,076	-	5,076	22,836
Total		5,076	-	5,076	22,836
Expenditure on:					
Raising funds	3	(255)	-	(255)	(257)
Charitable activities		(3,670)	-	(3,670)	(23,027)
Total		(3,925)	-	(3,925)	(23,284)
Net income/expenditure		1,151	-	1,151	(448)
Reconciliation of funds					
Total funds brought forward		31,232	3,417	34,649	35,097
Total funds carried forward		32,383	3,417	35,800	34,649

ACRODYSOSTOSIS SUPPORT & RESEARCH

Statement of Financial Position

As at 31 December 2023

	Notes	2023 £	2022 £
Current assets			
Debtors		431	207
Cash at bank and in hand		39,588	35,594
		40,019	35,801
Creditors: amounts falling due within one year	9	(4,219)	(1,152)
Net current assets		35,800	34,649
Total assets less current liabilities		35,800	34,649
Net assets		35,800	34,649
The funds of the charity			
Restricted income funds	10	3,417	3,417
Unrestricted income funds	10	32,383	31,232
Total funds		35,800	34,649

For the year ended 31 December 2023 the company was entitled to exemption from audit under section 477 of the Companies Act 2006 relating to small companies.

- The members have not required the company to obtain an audit of its accounts for the year in question in accordance with section 476,
- The trustees acknowledge their responsibilities for complying with the requirements of the Act with respect to accounting records and the preparation of accounts. These accounts have been prepared in accordance with the provisions applicable to companies subject to the small companies' regime.

The financial statements were approved and authorised for issue by the Board and signed on its behalf by:

DocuSigned by:

Mrs Nina Knight

25F8028178254B9

Nina Samantha Knight

Trustee

17 October 2024

ACRODYSOSTOSIS SUPPORT & RESEARCH

Notes to the Financial Statements For the year ended 31 December 2023

1. Accounting Policies

Basis of accounting

The financial statements have been prepared under the historical cost convention, except for investments which are included at market value and the revaluation of certain fixed assets and in accordance with the Charities SORP (FRS 102) 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), Financial Reporting Standard 102 the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102), and the (2011 or 2006 Act).

ACRODYSOSTOSIS SUPPORT & RESEARCH meets the definition of a public benefit entity under FRS 102. Assets and liabilities are initially recognised at historical cost or transaction value unless otherwise stated in the relevant accounting policy note(s).

The financial statements are prepared in sterling, which is the functional currency of the entity.

Going concern

The Trustees have assessed the charities finances and can confirm there is no reason why the charity will not continue to operate for at least 12 months passed the date of approval of these accounts.

Funds

Unrestricted funds are incoming resources receivable or generated for the furtherance of the objectives of the Charity without a specified purpose and are available as general funds. Resources expended which meet these criteria are charged to the funds, together with a fair allocation of staff and support costs. Restricted funds are used for the specific purposes laid down by the donor or sponsor. Resources expended which meet these criteria are charged to the funds.

Taxation

The Charity is exempt from taxation on its charitable activities, as it is a registered charity.

Debtors

Debtors are recognised at the settlement amount due after allowing for any discounts and as per the terms agreed. Prepayments are valued at the amounts prepaid for services and accrued income are valued at the amounts due per terms agreed and there is certainty of receipt.

Income and interest receivable

Income is recognised in the period when the charity has entitlement to the funds, any performance conditions attached to the income have been met, it is probable that the income will be received and that the amount can be measured reliably and is not deferred.

Income received in advance of the provision of a specified service is deferred until the criteria for income recognition are met.

Income comprises of funds received from donations as well as gift aid,

The value of services provided by volunteers has not been included.

Other accounting policies

Cash at bank and in hand:

Cash at bank and cash in hand includes bank balance kept in current and deposit accounts.

Creditors and provisions:

Creditors and provisions are recognised where the charity has a present obligation resulting from a past event that will probably result in the transfer of funds to a third party and the amount due to settle the obligation can be measured or estimated reliably. Creditors and provision are normally recognised at their settlement amount after allowing for any discounts.

Financial instruments:

The charity only has financial assets and financial liabilities of a kind that qualifies as basic financial instruments. Basic financial instruments are initially recognised at transaction value and subsequently measured at their amortised costs.

Critical accounting estimates and judgements:

There are no significant areas of judgement or key sources of estimation uncertainty arising from the preparation of accounts.

ACRODYSOSTOSIS SUPPORT & RESEARCH

Notes to the Financial Statements Continued

For the year ended 31 December 2023

2. Income from donations and legacies

	2023	2022
	£	£
Unrestricted funds		
Donations received	321	817
Gifts in kind	4,755	-
Donated goods and services	-	22,019
	5,076	22,836

3. Expenditure on generating donations and legacies

	2023	2022
	£	£
Unrestricted funds		
Support costs	255	257
	255	257

4. Analysis of support costs

	2023	2022
	£	£
Governance costs		
Accountancy fees	963	576
Subscriptions	2,707	432
Insurance	255	257
	3,925	1,265

It should be noted that subscriptions are higher this year as a backdated expense claim was made for 5 years of website costs. These costs had not previously been accrued for. In 2024 we expect subscription costs to be substantially lower.

Accountancy fees are higher this year due to an annual increase in fees as well as additional fees to claim for the gift aid refund which was received in the year.

ACRODYSOSTOSIS SUPPORT & RESEARCH

Notes to the Financial Statements Continued

For the year ended 31 December 2023

5. Net income/(expenditure) for the year

This is stated after charging/(crediting):

	2023	2022
	£	£
Accountancy fees	963	576
	<u> </u>	<u> </u>

6. Staff costs and emoluments

	2023	2022
General staff	0	0
	<u> </u>	<u> </u>
	0	0
	<u> </u>	<u> </u>

7. Trustee remuneration and related party transactions

There are no disclosable reimbursements to trustees in this financial period.

All amounts reimbursed in the year to trustees related to costs for fulfilling their duties as trustees as well as the reimbursement of costs which relate to the furtherance of the charity's objectives.

It should be noted that this year there was a backdated claim for subscriptions which had not previously been accrued for.

8. Comparative for the Statement of Financial Activities

The comparative year values on the Statement of Financial Activities are for unrestricted funds.

9. Creditors: amounts falling due within one year

	2023	2022
	£	£
Other creditors	3,499	576
Accruals and deferred income	720	576
	<u> </u>	<u> </u>
	4,219	1,152
	<u> </u>	<u> </u>

Included in other creditors is an amount due to the trustees for expense claims. This amount should be repaid within 12 months of the year end.

ACRODYSOSTOSIS SUPPORT & RESEARCH

Notes to the Financial Statements Continued

For the year ended 31 December 2023

10. Movement in funds

Unrestricted Funds

	Balance at 01/01/2023	Incoming resources	Outgoing resources	Balance at 31/12/2023
	£	£	£	£
<i>General</i>				
General	31,232	5,076	(3,925)	32,383
	<u>31,232</u>	<u>5,076</u>	<u>(3,925)</u>	<u>32,383</u>

Unrestricted Funds - Previous year

	Balance at 01/01/2022	Incoming resources	Outgoing resources	Balance at 31/12/2022
	£	£	£	£
<i>General</i>				
General	31,680	22,836	(23,284)	31,232
	<u>31,680</u>	<u>22,836</u>	<u>(23,284)</u>	<u>31,232</u>

Purpose of unrestricted Funds

General

To fulfil the principal objectives of the charity.

Restricted Funds

	Balance at 01/01/2023	Balance at 31/12/2023
	£	£
Family Day	3,417	3,417
	<u>3,417</u>	<u>3,417</u>

Restricted Funds - Previous year

	Balance at 01/01/2022	Balance at 31/12/2022
	£	£
Family Day	3,417	3,417
	<u>3,417</u>	<u>3,417</u>

ACRODYSOSTOSIS SUPPORT & RESEARCH
Notes to the Financial Statements Continued
For the year ended 31 December 2023

Purpose of restricted funds

Family Day

Delivering a family day for attendees as prescribed by the donors.

11. Analysis of net assets between funds

	Net current assets / (liabilities) £	Net Assets £
Unrestricted funds		
<i>General</i>		
General	32,383	32,383
Restricted funds		
Family Day	3,417	3,417
	35,800	35,800
Previous year		
	Net current assets / (liabilities) £	Net Assets £
Unrestricted funds		
<i>General</i>		
General	31,232	31,232
Restricted funds		
Family Day	3,417	3,417
	34,649	34,649

ACRODYSOSTOSIS SUPPORT & RESEARCH

Detailed Statement of Financial Activities

For the year ended 31 December 2023

	2023 £	2022 £
INCOME AND ENDOWMENT		
Donations and legacies		
Donations	321	817
Gifts in kind	4,755	-
Donated goods and services	-	22,019
	5,076	22,836
Total incoming resources	5,076	22,836
EXPENDITURE		
Charitable activities		
Roundtable research meeting costs (Roundtable research meetings)	-	(22,019)
	-	(22,019)
SUPPORT COSTS		
Governance costs		
Governance costs	(255)	(257)
Governance costs (Donation collections)	(3,670)	(1,008)
	(3,925)	(1,265)
Total resources expended	(3,925)	(23,284)
Net Income	1,151	(448)