



CHARITY COMMISSION
FOR ENGLAND AND WALES

Trustees' Annual Report for the period

From 01/04/2024

To 31/03/2025

Charity name: Cure DHDDS

Charity registration number: 1202643

Objectives and Activities

| | SORP reference | |
|--|--------------------|--|
| Summary of the purposes of the charity as set out in its governing document | Para 1.17 | The objects of the CIO are to relieve the needs of those affected by DHDDS Disease to support families affected by DHDDS mutations and drive research into them |
| Summary of the main activities in relation to those purposes for the public benefit, in particular, the activities, projects or services identified in the accounts. | Para 1.17 and 1.19 | <p>Families Support/Information Services</p> <p>The charity provides a support group for individuals and families affected by the condition. We aim to provide the latest information on the condition to families and health professionals via our website, e-news and social media.</p> <p>Educational Activities</p> <p>We continue to work on raising both public awareness and education amongst key medical professionals throughout the world so they can relate to the signs, symptoms and medical diagnosis of a DHDDS gene mutation.</p> <p>Speaking at and/or hosting exhibition stands (in-person or virtually) at medical and scientific conferences provides opportunities to fulfil our aims and objectives whilst developing strategic partnerships with research facilities, medical and pharmaceutical institutions, and like- minded charitable bodies.</p> <p>Research</p> <p>The charity has raised funds to pay for research to be undertaken by Sainte Justine Hospital, UCL Cardiff University and the ICAHN School of Medicine. There are a new research projects that we intend funding in 2025/26.</p> |

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| Statement confirming whether the trustees have had regard to the guidance issued by the Charity Commission on public benefit | Para 1.18 | The Trustees consider the charity's activities are wholly in the public interest and designed to benefit all those who suffer from DHDDS, their families and medical professionals dealing with this debilitating condition, in line with the Charity's Commission guidance on 'public benefit'. |
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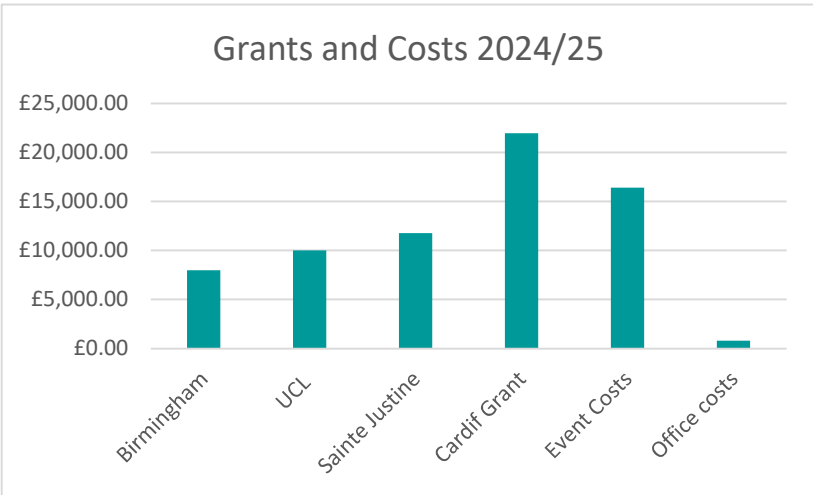
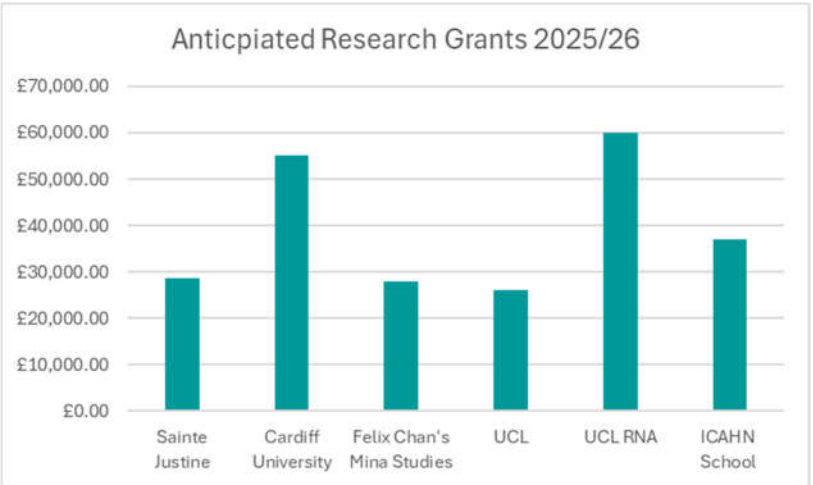
Achievements and Performance

| | SORP reference | |
|---|----------------|--|
| Summary of the main achievements of the charity, identifying the difference the charity's work has made to the circumstances of its beneficiaries and any wider benefits to society as a whole. | Para 1.20 | <p>Funding/Fundraising</p>  <p>We have once again been overwhelmed by the generosity that we have received from donors this year.</p> <p>Notable fundraising events included the John O'Groats to Lands' End Tandem Bike ride, the Home Guard DHDDS Summer Party, London Scottish Auction, the Midtown Business Club's Golf Day, Sophie Clegg's 3 Peak Challenge, Katie Barr's Brecon Beacon trek plus several independent fundraisers.</p> <p>Research</p> <p>Sainte Justine Hospital, Montreal – We supported a second year of Mouse model research with Alexey Pchejetski.</p> <p>University College London (UCL) – We funded preliminary research into an RNA therapy and subsequently Dr Raj Karda and Dr Ozge Cetin have applied for a grant to undertake AI-Based Preclinical Studies Utilizing AAV9-Mediated DHDDS-Specific Gene Therapy for Ultra-Rare DHHDS Disease.</p> <p>ICAHN School of Medicine – We have supported the development of IPSCS and DHDDS brain organoids for disease analysis and screening of NAD precursor treatments. This</p> |

| | | |
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| | | <p>has been translated into an international clinical trial under the supervision of Prof Eva Morava</p> <p>Cardiff University - Prof. Emyr Lloyd Evans and Dr Hannah Best – we have funded a 19 month research program focused on repurposing small molecule drugs developed for Niemann pick c disease in DHDDS patient cell models</p> <p>We continue to work very closely with Cure DHDDS USA on current and future research projects in the US including the development of Brain Organoids at Mount Sinai Hospital, the development of ASOs at n- Lorem and developing NAD precursors as potential therapies for DHDDS and NUS1 patients</p> <p>Conferences/Events attended + speaker opportunities: We hosted another two incredibly successful Cure DHDDS conferences in conjunction with Costello Medical. The very first conference was the winner of The Communique Awards 2024. There were 53 attendees at the Roundtable in April 2025 and then 70 attendees are expected at our Cure DHDDS Scientific Exchange Meeting in October 2025</p> <p>Mel has actively represented Cure DHDDS at numerous events in the last financial year both online and in person — to raise awareness of our mission and accelerate research. She has been featured in a range of podcasts, videos, and conferences, including:</p> <ul style="list-style-type: none"> • Behind the Genes Podcast – Genomics England • UNIAMO Podcast – Focus on DHDDS • Rare Disease Research Network • Patient Centricity Congress – One Nucleus • Festival of Genomics • Orphan Drugs Congress Europe 2024 • 7th Patient Centricity Congress 2025 • UPNAT Symposium • Genomics England Research Summit • Aspire Biosciences • N-Lorem Colloquium • Therapeutic Innovation and Trials – UCL • UK Platform for Nucleic Acid Therapies (UPNAT) Inaugural Symposium • Oxford Harrington Symposium |
|--|--|---|

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| | | <p>In addition to these events, Cure DHDDS has received media coverage from major outlets, including ITV London, <i>The Independent</i>, <i>Daily Mail</i>, <i>The Sun</i>, <i>Citywire</i>, and others — helping to significantly raise public awareness and accelerate research progress.</p> <p>Cure DHDDS has set up a patient registry with Cords at Sanford</p> <p>Cure DHDDS benefits from services provided by Costello Medical including mentoring, training, and logistical support</p> <p>We remain committed to supporting our rare disease community, many of whom have felt deeply isolated and underserved. Our private Facebook group now includes over 170 affected families, providing a safe and supportive space for connection and shared experiences.</p> <p>We have hosted several online meetings, bringing together both our international patient community and leading researchers to foster dialogue and collaboration. In addition, we regularly share research updates in both written and video formats, ensuring families stay informed and engaged.</p> |
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Financial Review

| Review of the charity's financial position at the end of the period | Para 1.21 | <p>Thanks to ongoing support from individuals and companies, the charity is in a strong position at the end of its second financial year.</p> <p>For the 12 months to the year end (31st March 2025) the charity had receipts of £131,495. Cash funds at the end of the financial year were £221,262.20.</p> <div><p>Grants and Costs 2024/25</p><table><thead><tr><th>Category</th><th>Amount (£)</th></tr></thead><tbody><tr><td>Birmingham</td><td>8,000</td></tr><tr><td>UCL</td><td>10,000</td></tr><tr><td>Sainte Justine</td><td>12,000</td></tr><tr><td>Cardiff Grant</td><td>22,000</td></tr><tr><td>Event Costs</td><td>16,000</td></tr><tr><td>Office costs</td><td>1,000</td></tr></tbody></table></div> <p>We have made four grants this financial year, with significant future commitments to Cardiff, Sainte Justine, UCL and Birmingham University as detailed below:</p> <div><p>Anticipated Research Grants 2025/26</p><table><thead><tr><th>Category</th><th>Amount (£)</th></tr></thead><tbody><tr><td>Sainte Justine</td><td>28,000</td></tr><tr><td>Cardiff University</td><td>55,000</td></tr><tr><td>Felix Chan's Mina Studies</td><td>28,000</td></tr><tr><td>UCL</td><td>25,000</td></tr><tr><td>UCL RNA</td><td>60,000</td></tr><tr><td>ICAHN School</td><td>38,000</td></tr></tbody></table></div> | Category | Amount (£) | Birmingham | 8,000 | UCL | 10,000 | Sainte Justine | 12,000 | Cardiff Grant | 22,000 | Event Costs | 16,000 | Office costs | 1,000 | Category | Amount (£) | Sainte Justine | 28,000 | Cardiff University | 55,000 | Felix Chan's Mina Studies | 28,000 | UCL | 25,000 | UCL RNA | 60,000 | ICAHN School | 38,000 |
|--|------------|--|----------|------------|------------|-------|-----|--------|----------------|--------|---------------|--------|-------------|--------|--------------|-------|----------|------------|----------------|--------|--------------------|--------|---------------------------|--------|-----|--------|---------|--------|--------------|--------|
| | Category | Amount (£) | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Birmingham | 8,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| UCL | 10,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Sainte Justine | 12,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Cardiff Grant | 22,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Event Costs | 16,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Office costs | 1,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Category | Amount (£) | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Sainte Justine | 28,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Cardiff University | 55,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Felix Chan's Mina Studies | 28,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| UCL | 25,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| UCL RNA | 60,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| ICAHN School | 38,000 | | | | | | | | | | | | | | | | | | | | | | | | | | | | | |
| Statement explaining the policy for holding reserves stating why they are held | Para 1.22 | <p>The purpose of the reserves is:</p> <ul style="list-style-type: none">➤ To fund working capital and time lags in the receipt of promised funds➤ To fund unexpected expenditure, e.g., when projects overrun, or unplanned events occur➤ Manage economic or regulatory changes which may have unforeseen financial consequences <p>We assess the level of general reserve needed by looking forward and considering the risks to our funding balanced against our expenditure commitments. To ensure we can continue to operate in accordance with our plans, we would look to hold a general reserve in the range £10,000 - £12,000 to cover unfunded committed costs for the next 6 months.</p> | | | | | | | | | | | | | | | | | | | | | | | | | | | | |

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| Amount of reserves held | Para 1.22 | Our reserves as of 31 March 2025 were £221,662 |
| Reasons for holding zero reserves | Para 1.22 | |
| Details of fund materially in deficit | Para 1.24 | |
| Explanation of any uncertainties about the charity continuing as a going concern | Para 1.23 | |

Additional information (optional)

You may choose to include further statements where relevant about:

| The charity's principal sources of funds (including any fundraising) | Para 1.47 | <div>Receipts 2024/25</div> <table><caption>Receipts 2024/25 Data</caption><tr><th>Source</th><th>Percentage</th></tr><tr><td>Corporate</td><td>20%</td></tr><tr><td>Individual</td><td>75%</td></tr><tr><td>Interest</td><td>5%</td></tr></table> | Source | Percentage | Corporate | 20% | Individual | 75% | Interest | 5% |
|--|------------|--|--------|------------|-----------|-----|------------|-----|----------|----|
| Source | Percentage | | | | | | | | | |
| Corporate | 20% | | | | | | | | | |
| Individual | 75% | | | | | | | | | |
| Interest | 5% | | | | | | | | | |
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Structure, Governance and Management

| | | |
|---|-----------|--|
| Description of charity's trusts: | | |
| Type of governing document (trust deed, royal charter) | Para 1.25 | CIO association constitution |
| How is the charity constituted? (e.g unincorporated association, CIO) | Para 1.25 | CIO association constitution (formed 06 April 2023) |
| Trustee selection methods including details of any constitutional provisions e.g. election to post or name of any person or body entitled to appoint one or more trustees | Para 1.25 | By appointment of registered members at general meetings and by Trustees |

Additional information (optional)

You may choose to include further statements where relevant about:

| | | |
|---|-----------|---|
| Policies and procedures adopted for the induction and training of trustees | Para 1.51 | New trustees are asked to abide by a Code of Conduct and are issued with Essential Information for Trustees from the Charities Commission, sign a Trustee Declaration and complete a Fit and Proper persons form. |
| The charity's organisational structure and any wider network with which the charity works | Para 1.51 | <p>Cure DHDDS is run on a voluntary basis.</p> <p>Trustees consult on all significant matters of policy formulation, family support and methods of communication about Cure DHDDS information services to medical professionals.</p> <p>The Scientific Advisory Board remains in place with 15 leading scientific advisors helping us understand the research and determine the most relevant use of funding.</p> <p>The charity operates the following policies: child protection, data privacy, risk management and volunteer management.</p> |
| Relationship with any related parties | Para 1.51 | <p>The charity is a member of:</p> <ul style="list-style-type: none"> ➤ Cure DHDDS and NUS 1 Community ➤ Genetic Alliance UK (GAUK) ➤ Rare X ➤ UK Rare Epilepsies Together network (UKRET) ➤ CDG Care ➤ CDG Global Alliance ➤ CDG UK ➤ LSD Collaborative • The Charity works closely with Cure DHDDS USA |

| | | |
|-------|--|--|
| | | |
| Other | | |

Reference and Administrative details

| | |
|-----------------------------|-------------------------------------|
| Charity name | Cure DHDDS |
| Other name the charity uses | |
| Registered charity number | 1202643 |
| Charity's principal address | 6 Park Avenue London SW14 8AT |
| | |

Names of the charity trustees who manage the charity

| | Trustee name | Office (if any) | Dates acted if not for whole year | Name of person (or body) entitled to appoint trustee (if any) |
|----|----------------|-----------------|-----------------------------------|---|
| 1 | Melanie Dixon | | | |
| 2 | Charles Dixon | | | |
| 3 | Victoria Allan | | | |
| 4 | Penny Brogan | | | |
| 5 | Louise Townley | | | |
| 6 | Katie McCombe | | | |
| 7 | | | | |
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| 19 | | | | |
| 20 | | | | |

Corporate trustees – names of the directors at the date the report was approved

[illegible]

Name of trustees holding title to property belonging to the charity

[illegible]

Funds held as custodian trustees on behalf of others

| | |
|---|--|
| Description of the assets held in this capacity | |
| Name and objects of the charity on whose behalf the assets are held and how this falls within the custodian charity's objects | |
| Details of arrangements for safe custody and segregation of such assets from the charity's own assets | |

Additional information (optional)

Names and addresses of advisers (Optional information)

| Type of adviser | Name | Address |
|-----------------|------|---------|
| | | |
| | | |
| | | |
| | | |

Name of chief executive or names of senior staff members (Optional information)

| |
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Exemptions from disclosure

Reason for non-disclosure of key personnel details

| |
|--|
| |
|--|

Other optional information

The Cure DHDDS Trustees would like to thank all their donors and volunteers for their support throughout the year.

Special mention should go to our DHDDS fundraising heroes during the period:

Ian Roberts

The Tandem team - Kenny Baillie, Piers Townley, Hamish Clegg, James Keen, Johnnie Wright, Mike Brogan, Matt Goodsall, Ian Roberts, Tim Dixon, Ashley Williams, Matt Lowe, Pete Harris, Nathan and Abi Brown, Will Hamilton, Andrew Paterson, Hugh Yarrow, Ben Peters

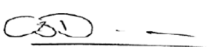
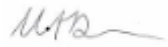
Katie Barr

Sophie Clegg

Declarations

The trustees declare that they have approved the trustees' report above.

Signed on behalf of the charity's trustees

| | | |
|-------------------------------------|---|--|
| Signature(s) |  |  |
| Full name(s) | Charles Dixon | Mel Dixon |
| Position (eg Secretary, Chair, etc) | Trustee | Trustee |
| Date | 04/12/2025 | |



CHARITY COMMISSION
FOR ENGLAND AND WALES

Cure DHDDS

No (if any)

Receipts and payments accounts

CC16a

For the period
from

01/04/2024

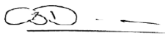

To

31/03/2025

Section A Receipts and payments

| | Unrestricted funds | Restricted funds | Endowment funds | Total funds | Last year |
|---|--------------------|------------------|------------------|------------------|------------------|
| | to the nearest £ | to the nearest £ | to the nearest £ | to the nearest £ | to the nearest £ |
| A1 Receipts | | | | | |
| Donations | £127,435.19 | 3,630 | - | 131,065 | - |
| Interest | 430 | - | - | 430 | - |
| | - | - | - | - | - |
| Sub total (Gross income for AR) | £127,865.30 | 3,630 | - | 131,495 | - |
| A2 Asset and investment sales, (see table). | | | | | |
| | - | - | - | - | - |
| | - | - | - | - | - |
| Sub total | - | - | - | - | - |
| Total receipts | 127,865 | 3,630 | - | 131,495 | - |
| A3 Payments | | | | | |
| Bank Fees | £85.00 | | | £85.00 | - |
| Computer for Mel | £630.98 | | | £630.98 | - |
| Event Merchandise | £4,081.66 | 1,605 | | £5,686.51 | - |
| Tandem Bike | £0.00 | 2,025 | | £2,025.00 | - |
| Website costs | £171.89 | | | £171.89 | - |
| Golf Event | £7,868.00 | | | £7,868.00 | - |
| Marathon | £480.00 | | | £480.00 | - |
| Hotel & Travel | £332.83 | | | £332.83 | - |
| Trade Body membership | £50.00 | | | £50.00 | - |
| Cardif Grant | £21,959.56 | | | £21,959.56 | - |
| UCL | £10,000.00 | | | £10,000.00 | - |
| Sainte Justine | £11,781.21 | - | - | £11,781.21 | - |
| Birmingham University | £8,000.00 | | | £8,000.00 | - |
| Sub total | £65,441.13 | £3,630 | - | 69,071 | - |
| A4 Asset and investment purchases, (see table) | | | | | |
| | - | - | - | - | - |
| Sub total | - | - | - | - | - |
| Total payments | 65,441 | 3,630 | - | 69,071 | - |
| Net of receipts/(payments) | 62,424 | - | - | 62,424 | - |
| A5 Transfers between funds | - | - | - | - | - |
| A6 Cash funds last year end | 159,238 | - | - | 159,238 | - |
| Cash funds this year end | 221,662 | - | - | 221,662 | - |

Section B Statement of assets and liabilities at the end of the period

| Categories | Details | Unrestricted funds to nearest £ | Restricted funds to nearest £ | Endowment funds to nearest £ |
|---|---|------------------------------------|----------------------------------|---------------------------------|
| B1 Cash funds | CAF Account | 221,566 | - | - |
| | | - | - | - |
| | | - | - | - |
| | Total cash funds | 221,662 | - | - |
| | (agree balances with receipts and payments account(s)) | OK | OK | OK |
| B2 Other monetary assets | Details | Unrestricted funds to nearest £ | Restricted funds to nearest £ | Endowment funds to nearest £ |
| | | - | - | - |
| | | - | - | - |
| | | - | - | - |
| | | - | - | - |
| | | - | - | - |
| | | - | - | - |
| B3 Investment assets | Details | Fund to which asset belongs | Cost (optional) | Current value (optional) |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| B4 Assets retained for the charity's own use | Details | Fund to which asset belongs | Cost (optional) | Current value (optional) |
| | Tandem Bike | | - | 2,025 |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| | | | - | - |
| B5 Liabilities | Details | Fund to which liability relates | Amount due (optional) | When due (optional) |
| | Sainte Justine | | £11,781.00 | |
| | Cardiff | | £23,776.00 | |
| | Icahn | | £35,200.00 | |
| | UCL | | £80,000.00 | |
| | Total | | £150,757.00 | |
| Signed by one or two trustees on behalf of all the trustees | Signature | Print Name | Date of approval | |
| |  | Charles Dixon | 04/12/2025 | |
| |  | Melanie Dixon | 04/12/2025 | |



Section A

Independent Examiner's Report

Report to the trustees

Charity Name
CURE DHDDS

On accounts for the year
ended

31 March 2025

Charity no
(if any)

1202643

Set out on pages

(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 March 2025.

Responsibilities and
basis of report

As the charity's trustees, 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 all the applicable Directions given by the Charity Commission under section 145(5)(b) of the Act.

Independent
examiner's statement

~~[The charity's gross income exceeded £250,000 and I am qualified to undertake the examination by being a qualified member of [insert name of applicable listed body]]. Delete [] if not applicable.~~

I have completed my examination. I confirm that no material matters have come to my attention in connection with the examination (~~other than that disclosed below~~ *) which gives me cause to believe that in, any material respect:

- the accounting records were not kept in accordance with section 130 of the Charities Act; or
- the accounts did not accord with the accounting records; or
- the accounts did not comply with the applicable requirements concerning the form and content of accounts set out in the Charities (Accounts and Reports) Regulations 2008 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.

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.

* Please delete the words in the brackets if they do not apply.

Signed:

Georgia Cragg

Date:

30/11/2025

Name:

Georgia Cragg

Relevant professional
qualification(s) or body

ICAEW

(if any):

Address:

Section B

Disclosure

Only complete if the examiner needs to highlight material matters of concern (see CC32, Independent examination of charity accounts: directions and guidance for examiners).

Give here brief details of any items that the examiner wishes to disclose.