



CHARITY COMMISSION  
FOR ENGLAND AND WALES

## Trustees' Annual Report for the period

From 31/03/2023

To 31/03/2024

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><b>Families Support/Information Services</b></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><b>Educational Activities</b></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><b>Research</b></p> <p>The charity has raised funds to pay for research to be undertaken by Perlara, Sainte Justine Hospital, UCL and new research with Cardiff and Aston University's is agreed with the intention of funding the research in 2024/25..</p>
Statement confirming whether the trustees have	Para 1.18	The Trustees consider the charity's activities

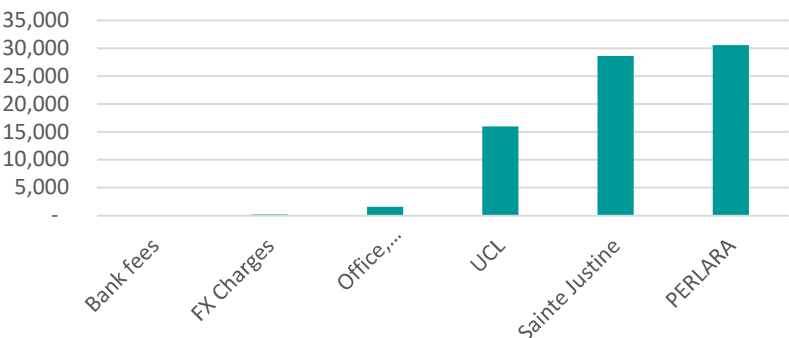
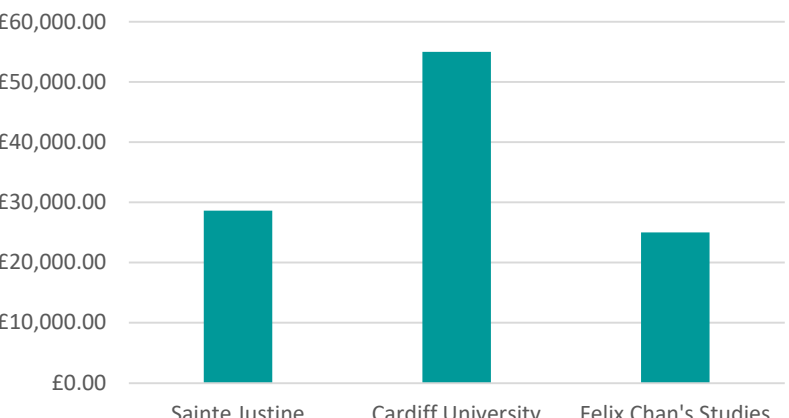
had regard to the guidance issued by the Charity Commission on public benefit		are wholly in the public interest and designed to benefit all those who suffer from DHDDS, their families and medical professionals dealing with this debilitating condition, in line with the Charity's Commission guidance on '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>In the first year since the charity was formed several aims were achieved:</p> <p><b>Funding/Fundraising</b></p>  <p>We have been overwhelmed by the generosity that we have received from donors in the first year.</p> <p>Notable member fundraising events included the 50km walk from Windsor to Mortlake, Will Gore's Royal Park run, London Scottish bucket shake, Zoe Dyer's lunch in Marbella, Susie Braam's coffee morning plus several independent fundraisers across the globe.</p> <p><b>Research</b></p> <p>Perlara, San Francisco – We funded a drug repurposing screen undertaken by Perlara. The Z prime experiment was successfully completed, and we have drug hits due to be tested in brain organoids at Mount Sinai Hospital.</p> <p>Sainte Justine Hospital, Montreal – We funded 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</p>

		<p>DHDDS-Specific Gene Therapy for Ultra-Rare DHHDS Disease.</p> <p>Cardiff University - Prof. Emyr Lloyd Evans and Dr Hannah Best – we have agreed to fund a 19 month research program focused on small molecule testing and NPC2 protein work. At a cost of £55,000</p> <p>Conferences/Events attended + speaker opportunities: We have hosted two incredibly successful Cure DHDDS conferences in conjunction with Costello Medical. The first of which has been nominated for The Communique Awards 2024. There were 30 attendees at the first Conference in September 2023 and then 18 at the second smaller roundtable meeting held in April 2024.</p> <p>Mel has presented at a number of events to raise the awareness of Cure DHDDS and the research we are undertaking, including:</p> <p>Beacon CAM Rare Festival of Genomics Orphan Drugs Congress 2024 Europe UPNAT symposium Genomics England Research Summit</p> <p>Cure DHDDS has set up a patient registry with Cords at Sanford</p> <p>We have also worked closely with Cure DHDDS USA on current and future research projects in the US including the development of Brain Organoids at Mount Sinai Hospital and novel iPSC cell line experiments at The Ready Centre UCSF.</p> <p>Cure DHDDS benefits from services provided by Beacon for rare diseases, CamRARE including mentoring, training, and networking opportunities.</p>
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## Financial Review

<p>Review of the charity's financial position at the end of the period</p>	<p>Para 1.21</p>	<p>The charity is in a very fortunate position at the end of the first financial year as a result of the generosity of many individuals and companies.</p> <p>To the year end (31<sup>st</sup> March 2023) the charity had receipts of £236,221.</p> <div><p>Grants and Costs 23/24</p><table><thead><tr><th>Category</th><th>Value</th></tr></thead><tbody><tr><td>Bank fees</td><td>0</td></tr><tr><td>FX Charges</td><td>0</td></tr><tr><td>Office,...</td><td>1,000</td></tr><tr><td>UCL</td><td>15,000</td></tr><tr><td>Sainte Justine</td><td>28,000</td></tr><tr><td>PERLARA</td><td>30,000</td></tr></tbody></table></div> <p>We have made three grants thus far as detailed under research, with significant future commitments to Cardiff, Dr Felix Chan and Sainte Justine as detailed below:</p> <div><p>2024/25 Research Grants</p><table><thead><tr><th>Category</th><th>Value</th></tr></thead><tbody><tr><td>Sainte Justine</td><td>£28,000.00</td></tr><tr><td>Cardiff University</td><td>£55,000.00</td></tr><tr><td>Felix Chan's Studies</td><td>£25,000.00</td></tr></tbody></table></div>	Category	Value	Bank fees	0	FX Charges	0	Office,...	1,000	UCL	15,000	Sainte Justine	28,000	PERLARA	30,000	Category	Value	Sainte Justine	£28,000.00	Cardiff University	£55,000.00	Felix Chan's Studies	£25,000.00
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Cardiff University	£55,000.00																							
Felix Chan's Studies	£25,000.00																							
<p>Statement explaining the policy for holding reserves stating why they are held</p>	<p>Para 1.22</p>	<p>The purpose of the reserves is:</p> <ul style="list-style-type: none"><li>➤ To fund working capital and time lags in the receipt of promised funds</li><li>➤ To fund unexpected expenditure, e.g., when projects overrun, or unplanned events occur</li><li>➤ Manage economic or regulatory changes which may have unforeseen financial consequences</li></ul> <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>																						

Amount of reserves held	Para 1.22	Our reserves as of 31 March 2024 were £159,738
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 through 2023/24</div> <table><tr><th>Source</th><th>Amount (£)</th></tr><tr><td>Corporate Donations</td><td>85,000.00</td></tr><tr><td>Individual Donations</td><td>150,000.00</td></tr><tr><td>Bank Interest</td><td>5,000.00</td></tr></table>	Source	Amount (£)	Corporate Donations	85,000.00	Individual Donations	150,000.00	Bank Interest	5,000.00
Source	Amount (£)									
Corporate Donations	85,000.00									
Individual Donations	150,000.00									
Bank Interest	5,000.00									

## 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 10 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, published on our website.</p>
Relationship with any related parties	Para 1.51	<p><b>The charity is a member of:</b></p> <ul style="list-style-type: none"> <li>➤ Cure DHDDS and NUS 1 Community</li> <li>➤ Genetic Alliance UK (GAUK)</li> <li>➤ Rare X</li> <li>➤ UK Rare Epilepsies Together network (UKRET)</li> <li>➤ CDG Global Alliance</li> <li>➤ CDG UK</li> <li>• The Charity works closely with Cure DHDDS USA</li> </ul>

Other		
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## Reference and Administrative details

Charity name	Cure DHDDS
Other name the charity uses	
Registered charity number	<b>1202643</b>
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				
8				
9				
10				
11				
12				
13				
14				
15				
16				
17				
18				
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

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## 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:

Freddie Tidbury

Sophie Clegg

Will Gore

Zoe Dyer

Susie Braam



## 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)		
Position (eg Secretary, Chair, etc)		
Date		



CHARITY COMMISSION  
FOR ENGLAND AND WALES

Charity Name  
Cure DHDDS

No (if any)  
1202643

## Receipts and payments accounts

CC16a

For the period  
from

Period start date  
4/1/2023

To

Period end date  
3/31/2024

### 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 £
<b>A1 Receipts</b>					
Donations & Fundraising activities	235,972	-	-	235,972	-
Bank Interest	249	-	-	249	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
<b>Sub total</b> (Gross income for AR)	236,221	-	-	236,221	-
<b>A2 Asset and investment sales, (see table).</b>					
	-	-	-	-	-
	-	-	-	-	-
<b>Sub total</b>	-	-	-	-	-
<b>Total receipts</b>	236,221	-	-	236,221	-
<b>A3 Payments</b>					
Bank fees	50	-	-	50	-
Sainte Justine	28,620	-	-	28,620	-
PERLARA	30,570	-	-	30,570	-
FX Charges	175	-	-	175	-
UCL	16,000	-	-	16,000	-
Office, Subscriptions and Events	1,568	-	-	1,568	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
<b>Sub total</b>	76,983	-	-	76,983	-
<b>A4 Asset and investment purchases, (see table)</b>					
	-	-	-	-	-
	-	-	-	-	-
<b>Sub total</b>	-	-	-	-	-
<b>Total payments</b>	76,983	-	-	76,983	-
<b>Net of receipts/(payments)</b>	159,238	-	-	159,238	-
<b>A5 Transfers between funds</b>	-	-	-	-	-
<b>A6 Cash funds last year end</b>	-	-	-	-	-
<b>Cash funds this year end</b>	159,238	-	-	159,238	-

## 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 £
<b>B1 Cash funds</b>	CAF account	159,738	-	-
		-	-	-
		-	-	-
	<b>Total cash funds</b>	159,738	-	-
	(agree balances with receipts and payments account(s))	Agreement Error	OK	OK
<b>B2 Other monetary assets</b>	Details	to nearest £	to nearest £	to nearest £
		-	-	-
		-	-	-
		-	-	-
		-	-	-
		-	-	-
		-	-	-
<b>B3 Investment assets</b>	Details	Fund to which asset belongs	Cost (optional)	Current value (optional)
			-	-
			-	-
			-	-
			-	-
<b>B4 Assets retained for the charity's own use</b>	Details	Fund to which asset belongs	Cost (optional)	Current value (optional)
			-	-
			-	-
			-	-
			-	-
			-	-
			-	-
			-	-
			-	-
<b>B5 Liabilities</b>	Details	Fund to which liability relates	Amount due (optional)	When due (optional)
			-	
			-	
			-	
			-	
Signed by one or two trustees on behalf of all the trustees	Signature	Print Name	Date of approval	



Section A

Independent Examiner's Report

Report to the trustees

Charity Name  
CURE DHDDS

On accounts for the year  
ended

31 March 2024

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 / 03 / 2024 .

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:

Date:

23 August 2024

Name:

Georgia Cragg

Relevant professional  
qualification(s) or body

ICAEW

(if any):

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Address:

Loughrigg, Sunnyside Close, Lancaster, LA1 5NH

**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.

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