



## Trustees' Annual Report for the period

	Period start date				Period end date		
<b>From</b>	01	06	2020	<b>To</b>	31	05	2021

Charity name

Other names charity is known by

Registered charity number (if any)

Charity's principal address

**Postcode**

### 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 Mark Leverington	Secretary		
2 Russell Wheeler			
3 James Ferguson			
4 Charlotte McMillan			
5			
6			
7			
8			
9			
10			

### Names of the trustees for the charity, if any, (for example, any custodian trustees)

Name	Dates acted if not for whole year

**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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**Description of the charity's trusts**

Type of governing document (eg. trust deed, constitution)	Constitution, dated 16 Jan 2017
How the charity is constituted (eg. trust, association, company)	Charitable Incorporated Organisation – registered 28 May 2014
Trustee selection methods (eg. appointed by, elected by)	By existing trustees. Minimum of three, maximum of twelve trustees

**Additional governance issues (Optional information)**

You **may choose** to include additional information, where relevant, about:

- policies and procedures adopted for the induction and training of trustees;
- the charity's organisational structure and any wider network with which the charity works;
- relationship with any related parties;
- trustees' consideration of major risks and the system and procedures to manage them.

The society is a full and active member of EURORDIS, a non-governmental patient-driven alliance of patient organisations representing 724 rare disease patient organisations in 64 countries covering over 4000 diseases ([www.eurordis.org](http://www.eurordis.org)). Through this membership the society aims to be kept informed of policy changes and developments throughout Europe which affect rare disease patients and in conjunction with EURORDIS to influence those policies wherever possible for the benefit of our members and rare disease patients in general.

The society continues to hold discussions with other LHON patient representatives from other countries in Europe, with a view to establishing more formal links between us in order to collaborate on research and understanding of the disease.

The society continues to participate in the European Reference Network for rare eye diseases ("ERN-EYE") a European Union initiative which brings together leading specialists from 28 different specialist ophthalmic centres from 13 different member states in order to collaborate on research and treatment of rare eye diseases.

**Summary of the objects of the charity set out in its governing document**

1. To relieve the charitable needs of people affected by LHON, their families and carers by the provision of support, advocacy, advice and information
2. To carry out, or to provide funds to support, research in all aspects of LHON: its causes, treatment and cure. To publish the useful results of such research

We are a self-help support group for families affected by LHON across the British Isles. Through our membership we aim to identify as many affected families as possible and build a register of people willing to take part in medical research. We provide up-to-date information on LHON and current research topics.

**Summary of the main activities undertaken for the public benefit in relation to these objects (include within this section the statutory declaration that trustees have had regard to the guidance issued by the Charity Commission on public benefit)**

**Additional details of objectives and activities (Optional information)**

You **may choose** to include further statements, where relevant, about:

- policy on grantmaking;
- policy programme related investment;
- contribution made by volunteers.

**Summary of the main achievements of the charity during the year**

Last year's report of activity (to May 2020) just reached into the early days of the Covid crisis and we reported then on how some of our funding requests had been already damaged by the understandable need to divert resources to the pandemic response and unfortunately this remained a theme throughout the year, as indeed did the crisis itself. Plans for a patient meeting in the autumn were of course cancelled and all activity switched to virtual platforms. This has been a learning curve for everyone and it seems likely that such virtual platforms will remain a part of our communications armoury going forward, although hopefully supplemented by face to face meetings as and when these are possible.

Thus the society's AGM was held on 4th July as a completely virtual event over Zoom and this was well attended and surprisingly effective, with a lively discussion and in addition to the formal AGM itself we successfully held a mini version of what we might have delivered if we had been able to meet in person. We are getting better at this, and we can hopefully learn from this activity which has been forced upon us to better communicate with those who are unable to attend our in-person meetings for whatever reason.

The effects of the pandemic were felt everywhere in society and in the research area especially the vast majority of non-Covid projects were at best severely delayed and in many cases abandoned. It had been our aim to sponsor a networking event in September to bring together a wide range of researchers from different fields all over Europe who have an interest in LHON or in vision impairment generally or mitochondrial or other related disease but would not normally meet together. This is all too common in research, which develops in silos with the end result that lots of work is duplicated and lessons learned are not transferable and mistakes are repeated. Unfortunately, due to the cancellation of all face to face meetings this had to be abandoned (it was to have piggy-backed on a vision research meeting which was also cancelled) as it is impractical to kick-start such a novel approach via a virtual presentation. The concept remains every bit as attractive, if not more so, and we will resurrect this as soon as we are able to do so.

Notwithstanding the effects of the pandemic, the only approved treatment for LHON, Raxone, was assessed by the All Wales Therapeutics and Toxicology Centre (AWTTC) on behalf of the NHS in Wales and, despite an initial rejection, this was reviewed in January 2021 and following representations from LHON Society and strong testimony from some individual society members in Wales, the decision was reversed and Raxone is now available on prescription in Wales. A further review also took place in England but we were not consulted by NHS England on this (despite pushing) and in a decision that was just as opaque and difficult to understand as their initial rejection they held fast in their refusal to grant access to this treatment. As a result, England is the only country in the British Isles where Raxone is not available on prescription and, indeed, in most of Europe.

The clinical trials for gene therapy as treatment for the ND4 mutation in LHON undertaken by French biotech company Gensight came to a successful conclusion during the year and application was made to the European Medicines Agency (EMA) for approval. The review of this treatment is taking much longer than anticipated, also in part impacted by the Covid crisis, and the outcome is still unknown at the time of writing this report.

Regrettably, at the end of the transition period for the UK's departure from the EU, the four UK University hospital centres (Moorfields, Oxford, Manchester and Leeds) had to leave the European Reference Network for Rare Eye Diseases ([www.ern.eye.eu](http://www.ern.eye.eu)) but contacts built up through this network will be maintained as closely as possible and LHON Society remains on the governing board of this important network dedicated to improving research and outcomes for all affected by rare eye diseases. The world of rare diseases is small enough already, without politics making it smaller. One of the most important projects for this network is the establishment of a Europe-wide registry for all rare eye diseases (REDgistry) which will contain data elements that allow for information to be grouped on an anonymous basis to support research and enable accelerated progress towards better understanding of diseases and development of novel treatments. This is not the same as a specific disease registry, which remains our ultimate goal, where the specific data for LHON is captured and available for analysis, but it is a vital element in building the right foundations for a LHON registry that is truly compatible internationally – right now there are LHON registries in some European countries (not in the UK) but it is not possible to aggregate information and their reporting is inconsistent. In such a rare disease, the benefits of a small national register are very limited and we must be able to scale this up to extract the full benefits.

The information generated by a strong disease registry and natural history study both form part of what in the jargon is termed Real World Data or RWD. This in turn forms part of Real World Evidence (RWE) which is increasingly being recognised by regulators and payers worldwide as an important element in determining the efficacy, safety and, ultimately, value of a medical treatment for any given disease. An over-reliance on clinical trial data in the past has been a major cause of delays and setbacks in the development of treatments for rare disease and we welcome this more enlightened approach to supplementing and, in some cases, even replacing clinical trial data.

The emergence of new techniques and tools for analysing data (so-called Artificial Intelligence and Machine Learning) makes all of this much more possible now, together with plummeting costs of computing power. LHON Society is supporting EMA in their efforts to move in this direction through membership of their Advisory Group on Raw Data and we are in discussion with several researchers seeking to use these tools in order to identify new treatment options, especially amongst those already approved for other diseases. Raxone itself is a repurposed drug that was developed for another disease but the pathway to its use in LHON took several years and these efforts are aimed at finding even better compounds in a much shorter time frame. Although the details are confidential, some compounds have already been identified and are under examination as a result of this approach.

Given the potential value of these techniques to the LHON community and the importance of high quality data in order to fuel their application, you can expect to hear a lot more about our need for developing that data in the coming years.

**Brief statement of the charity's policy on reserves**

Considering its relatively low levels of income and expenditure (see below), the trustees do not require any reserves to be held by the charity

**Details of any funds materially in deficit**

None

**Further financial review details (Optional information)**

You **may choose** to include additional information, where relevant about:

- the charity's principal sources of funds (including any fundraising);
- how expenditure has supported the key objectives of the charity;
- investment policy and objectives including any ethical investment policy adopted.

Financial Report as at 31/05/2021

Opening balance brought forward: £19238

Income during the year:

£1108 – exclusively from fundraising and donations

Expenditure:

£80 - charity bank account fees

£43 - EURORDIS membership fee

Closing Balance at 31/05/2019: £20223

**Section F**

**Other optional information**

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

Signed on behalf of the charity's trustees

Signature(s)



Full name(s)

Mark Leverington

Position (eg Secretary, Chair, etc)

Trustee & Secretary



**Date** 17<sup>th</sup> March 2022



Charity Name		No (if any)		CC16a
Leber's Hereditary Optic Neuropathy Society		1157206		
Receipts and payments accounts				
For the period from	Period start date	To	Period end date	
	01/06/2020		31/05/2021	


## 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>					
Exclusively from donations	1,108	-	-	1,108	8,499
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
Sub total(Gross income for AR)	1,108	-	-	1,108	8,499
<b>A2 Asset and investment sales, (see table).</b>					
	-	-	-	-	-
	-	-	-	-	-
Sub total	-	-	-	-	-
<b>Total receipts</b>	1,108	-	-	1,108	8,499
<b>A3 Payments</b>					
	-	-	-	-	-
	-	-	-	-	-
Bank account fees	80	-	-	80	60
Genetic Alliance UK membership fee	-	-	-	-	50
Mailchimp account fees	-	-	-	-	17
EURORDIS membership fee	43	-	-	43	43
	-	-	-	-	-
	-	-	-	-	-
	-	-	-	-	-
Sub total	123	-	-	123	170
<b>A4 Asset and investment purchases, (see table)</b>					
	-	-	-	-	-
	-	-	-	-	-
Sub total	-	-	-	-	-
<b>Total payments</b>	123	-	-	123	170
<b>Net of receipts/(payments)</b>	985	-	-	985	8,329
<b>A5 Transfers between funds</b>	-	-	-	-	-
<b>A6 Cash funds last year end</b>	19,238	-	-	19,238	-
<b>Cash funds this year end</b>	20,223	-	-	20,223	8,329

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

Categories	Details	Unrestricted funds	Restricted funds	Endowment funds
		to nearest £	to nearest £	to nearest £
<b>B1 Cash funds</b>		-	-	-



		-	-	-
		-	-	-
	Total cash funds	20,223	-	-
	(agree balances with receipts and payments account(s))	OK	OK	OK
		Unrestricted funds	Restricted funds	Endowment funds
	Details	to nearest £	to nearest £	to nearest £
B2 Other monetary assets		-	-	-
		-	-	-
		-	-	-
		-	-	-
		-	-	-
		-	-	-
	Details	Fund to which asset belongs	Cost (optional)	Current value (optional)
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 liability relates	Amount due (optional)	When due (optional)
B5 Liabilities			-	
			-	
			-	
			-	
			-	
Signed by one or two trustees on behalf of all the trustees	Signature	Print Name		Date of approval
		Mark Leverington		27/01/22