

# BARTH SYNDROME UK

## 2023 Annual Report



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## **BARTH SYNDROME UK**

### **TRUSTEES' REPORT**

The trustees present their report for the year ended 31 December 2023.

### **General Information**

**REGISTERED CHARITY NUMBER:** 1181830

**TRUSTEES:** Mr Ralph Easterbrook – Chairperson  
Mr Geoffrey Parish – Treasurer  
Mrs Kelsey Doran – Trustee  
Mrs Isabel Easterbrook – Trustee  
Mr Stephen Cotterill – Trustee  
Mrs Cheryl Parish - Trustee  
Mrs Suzanne Green – Trustee  
Ms Kate Riseborough-Evans (resigned 26<sup>th</sup> July 2023)

**STAFF:** Mrs Michaela Damin - Chief Executive

**REGISTERED OFFICE:** 1 The Vikings  
Romsey  
Hampshire  
SO51 5RG

### **STATEMENT OF TRUSTEES' RESPONSIBILITIES**

The trustees are required to prepare accounts for each financial year, which reflect the receipts and payments of the charity and of the surplus or deficit of income against payments for the year.

The trustees are responsible for:

- keeping proper accounting records which disclose with reasonable accuracy at any time the financial position of the charity; and
- safeguarding the assets of the charity and hence for taking reasonable steps for prevention and detection of fraud and other irregularities.

### **LEGAL AND ADMINISTRATIVE**

The charity was registered on 31<sup>st</sup> January 2019 as a Charitable Incorporated Organisation (CIO) with the Charity Commission. The charity is governed by a CIO - Foundation Governing document.

## RESERVES POLICY

Barth Syndrome UK (BS UK) requires a level of free reserves to safeguard the organisation, protect options for future development and to achieve the following: -

- The option to develop new services or expand current ones in line with the changing needs of the community.
- To ensure that shortfalls in expected income, or delays in the receipt of expected income, do not interrupt services, or prevent the payment of salaries, or cause financial difficulty for the charity.
- The ability to survive unexpected setbacks and problems arising from internal or external causes.

The **minimum** level of such reserves should be at least equivalent to the expected outgoings for a period of 12 months. Should the level of reserves fall below or be expected to fall below this level, the Board of Trustees will develop a plan to restore the level of reserves.

The **maximum** level of such reserves should be no greater than the expected outgoings for a period of 24 months. Should the level of reserves be higher than this level, the Board of Trustees will develop a plan to allocate these funds towards agreed program goals.

## What is Barth Syndrome?

Barth syndrome is a rare, life-threatening, genetic mitochondrial disorder primarily affecting boys and men. Affected people may suffer from heart failure, muscle weakness, and infection (caused by neutropenia).

Additional characteristics of the syndrome commonly include growth delay, impaired lipid metabolism, debilitating fatigue, and cardiolipin deficiency.

For some people with Barth syndrome, symptoms can be very severe, sometimes resulting in heart transplant, potentially lethal infections, and even death.

**OUR VISION:** A world in which people with Barth syndrome live, and live well.

**OUR MISSION:** Saving lives through education, advances in treatment and finding a cure for Barth syndrome.

## OBJECT(S)

The objects[s] of the CIO are:

The relief of individuals with, and carriers of, Barth syndrome and their families, within the United Kingdom and elsewhere in the world in particular but not exclusively by:

a) providing access to information, advice and support in relation to the condition to the public and, in particular, diagnosed individuals and their extended families and those in the educational, scientific, medical and allied health care professions.

b) the promotion and funding of research into the causes and effects of the condition and the dissemination of the useful results of the same for the benefit of the public.



## INTRODUCTION FROM THE CHAIR

Another challenging year for the charity; as always, the constant need for funds weighed heavy, but the concern was lightened somewhat by being the chosen charity for Barratt's and David Wilson Homes, to whom we are eternally grateful. Many thanks and huge appreciation to Shea and Kelsey Doran, who identified and made the best possible use of this opportunity.

A successful weekend was held at Penny Bohn, Bristol, disseminating the latest medical advances and research opportunities, and perhaps more

importantly, enabling families to connect and engage after the Covid-19 crisis. The feedback was almost entirely positive, and hopefully other similar events can be organised and held in the near future.

Our young adult cohort continue to meet and interact via social media, zoom etc. and now also in face-to-face gatherings funded by the charity; we feel it is important that those affected can meet and share their experiences with their peers in a safe and welcoming arena. These young men (almost all affected are males) also act as role models for the families of young infants diagnosed with Barth Syndrome – I know from personal experience that in the dark times it is especially important to have hope and to know that it is possible not just to survive but actually thrive.

However, the charity is also here to care for, support and help those who are going through a medical crisis or suffer the loss of a loved one; we have a wealth of information built up over the years to help families and medical practitioners, as well as a family support section available to any who require assistance.

Ralph Easterbrook  
Chair, Barth Syndrome UK

## MESSAGE FROM THE CHIEF EXECUTIVE, Michaela Damin:

Thank you to every person who supported us in 2023 as we continued to provide our vital services.



We are incredibly fortunate to have the support of a dedicated NHS Barth Syndrome Service, a passionate and committed Board of Trustees and a hardworking band of volunteers, families and friends, all keeping us strong and positive.

All of us at Barth Syndrome UK pledge to keep working hard in 2024 to serve our affected families and make a positive difference in their lives.

*Michaela Damin*

## REVIEW OF ACTIVITIES

### SUPPORTING FAMILIES

#### ONE ON ONE SUPPORT FOR FAMILIES AND INDIVIDUALS

Much of our day-to-day work consists of supporting individuals from pre-diagnosis onwards. This is done in person, by telephone and via private online forums.

BS UK offers affected families financial help via a Transport Costs Scheme which allows families to claim back their travel expenses when attending a specialist Barth syndrome clinic in Bristol.

In 2023 the Board voted to extend this help by also offering families the ability to claim back a meal when clinic attendance requires an overnight stay.

#### GROUP SUPPORT AND INFORMATION

##### NATIONAL WEEKEND MEETING: 15<sup>TH</sup>-16<sup>TH</sup> APRIL 2023

On Sat 15th April 2023, 19 researchers and health professionals from around the world met with 51 family members at the beautiful Penny Brohn Centre near Bristol.

Thanks to funding from the NIHR, Barth Syndrome UK was able to organise this national gathering to share the results of the CARDIOMAN clinical trial, the first ever trial in Barth syndrome in the UK.

The weekend kicked off on Friday with dinner and an evening of catching up over some fun games such as bingo whilst everyone wound down from travelling.

On Saturday we hosted informative talks and presentations from leading researchers and medical professionals around various topics, followed by an entertaining quiz enjoyed by those of all ages.

Sunday was spent learning new skills like archery and laser clay pigeon shooting and carers were offered massages and facial treatments for some rare pamper time.

After the isolation brought about by Covid, this weekend was a chance for our Barth community to re-connect and to feel inspired by the hope of new research and treatments for this life-limiting condition.

Feedback after the event was collected via a survey and the feedback received was overwhelmingly positive, with 100% of respondents rating the event as Excellent, Very Good or Good.

#### YOUNG ADULTS AND MEN PROJECT FUNDED BY JEANS FOR GENES GRANT

Our babies are growing up. And that's always a great sign in a rare, life-limiting condition because it means that advances in medicine are resulting in a longer life for many in our community.

Thanks to a grant from Jeans for Genes, BS UK was able to organise specialist training for two of our young men to act as mentors and facilitators. After training was completed, the two young men set up weekly online meetings for affected individuals 16 yrs+ to provide peer-to-peer support and a chance to meet socially. These meetings have proved to be popular and are ongoing. A face-to-face meeting was also organised by the group members towards the end of the year with everyone meeting up in Bristol in November 2023.

## **COUNSELLING & COGNITIVE HYPNOTHERAPY**

Having Barth syndrome unsurprisingly results in many additional mental health issues for affected individuals and their families. In response to a lack of access to counselling and psychological support, BS UK partnered with Longcroft Therapy to start a pilot project, offering 1:1 counselling sessions to those in need.

This project has been a huge success, enabling family members to deal more effectively with the challenges that are faced on a day-to-day basis and to consider alternative mindsets to manage the long-term anxieties and quality of life issues that so often accompany lifelong medical interventions in a genetic condition.

Plan to grow this pilot scheme are in place for 2024.

## **BRISTOL NHS SPECIALISED SERVICE**

We continue to send a representative from the charity to every monthly Barth syndrome clinic in Bristol to ensure that patients' voices are heard and to make sure that we can ask what our families need from us. We also reimburse families' travel costs to make the clinic accessible to all. Families travel from as far as Scotland to attend specialist clinics.

## **RAISING AWARENESS**

### **RARE DISEASE DAY AND BRITISH PAEDIATRIC SURVEILLANCE UNIT (BPSU)**

Kelsey Doran (Trustee and mother of an affected child) travelled to London to raise awareness for Rare Disease Day at the BPSU annual meeting, accompanied by Barth Syndrome Service Specialist Nurse, Gillian Alexander. They provided an overview of Barth syndrome, both from a medical and from a family point of view and their presentations resulted in increased requests for testing from medical professionals in the audience.

### **IMPROVED COMMUNICATIONS**

In 2023 we set out to improve our social media presence and to improve communications via shorter news blasts to our community. With a dedicated volunteer running this project, we're seeing a growth in our online presence, and we have plans to continue this growth in 2024.

### **WEBSITE REDESIGN**

Our current website continues to be an excellent source of quality information. However, since it was designed over twelve years ago, the Board voted to redesign it to modernise it and to make it mobile friendly. Work will commence in 2024 on this new project.

## RESEARCH AND MEDICAL PROGRAMS

### Funding Research

Barth Syndrome UK, along with Barth Foundation Canada, co-funded the following research project in 2023:

#### **Investigating the basis of neutropenia in Barth Syndrome**

**Borko Amulic**, Professor, University of Bristol

This research project - led by Profs Borko Amulic and Colin Steward in Bristol, UK - hopes to increase our understanding of neutropenia in Barth syndrome, i.e. **why people with Barth syndrome have neutropenia and why their neutrophil counts fluctuate so much at times.**

### INTERNATIONAL REGISTRY

We continued to encourage families to sign up to our [International Registry and Biobank](#) in our efforts to promote research.

### INTERNATIONAL CONFERENCE

Work started in 2023 as we plan the International Barth Syndrome Conference which will be held in Bonita Springs, Florida in July 2024. This is a joint Family, Scientific and Medical conference and it attracts the largest number of affected families and experts in the world.

### ASPIRE BIOSCIENCES LONDON

In December 2023, BS UK was fortunate to attend the Aspire Biosciences Industry Partnering Event at the Royal Society of Medicine in London.

This was a very well organised and productive event, and its aim was to partner patient group charities with biotech and pharma industries, with a view to accelerating advances in therapies for patients.

Michaela Damin, Chief Executive and Dr Germaine Pierre (Co Lead for the NHS Barth Syndrome Service) presented at the event and their attendance has led to some successful and ongoing collaborations.

### NHS ENGLAND GENERATIONAL STUDY

Barth syndrome has been chosen to be included in the upcoming [Generation Study](#)

The Generation Study is an NHS-embedded research study which will explore the benefits, challenges and practicalities of sequencing and analysing newborns' genomes. The study will sequence and analyse the genomes of 100,000 newborn babies in the UK. It is a hybrid clinic-research study that aims to generate evidence on whether whole genome sequencing can be used to screen newborns for rare genetic conditions, and to assess the feasibility of doing this within the NHS.

## STRATEGIC PLANNING

The Board of Trustees and the Chief Executive met for an in-person weekend planning session in July 2023 with a view to creating an updated Fundraising Strategy and to grow the Board. This resulted in an effective blueprint for activities for the second half of the year. In addition, the board meets on a monthly basis to ensure effective running of the charity.

## FUNDRAISING

We are hugely grateful to all our supporters in 2023. Without your donations and fundraising, we would not exist.

The charity received £2190 from regular standing orders from families and supporters, gift aid of £1837 and bank interest of £614.

Fundraising activities and events brought in £13685; special mention to Mark Wass, who raised £2415 running the London Marathon, and the Doran family who raised £4226 taking part in a mud racing event. Other events included car boot sales, sales of Christmas merchandise, and a number of funeral collections.

By far the largest amounts received during the year were via grants and/or donations received from Barratt David Wilson Eastern Counties (£41 946), Jeans for Genes (£3 000) & Cardioman Research Project grant (£16 100).

## BARTH SYNDROME UK

### Receipts and Payments Account for the period 1<sup>ST</sup> January 2023 to 31 December 2023

|  | Notes | 2023<br>Total<br>£ | 2022<br>Total<br>£ |
|--|-------|--------------------|--------------------|
| <b>RECEIPTS</b>                                    |       |                    |                    |
| Fundraising events                                 |       | -                  | 5,291              |
| Gifts and donations                                |       | 76,921             | 5,305              |
| Gift Aid claims                                    |       | 1,838              | 1,093              |
| Interest received                                  |       | 615                | 156                |
| <b>Total receipts</b>                              |       | <b>79,374</b>      | <b>11,845</b>      |
| <b>PAYMENTS</b>                                    |       |                    |                    |
| Group development                                  |       | 698                | 784                |
| Fundraising expenses                               |       | 256                | 2,080              |
| Science and medicine                               |       | 20,417             | 14,991             |
| Family support and education                       |       | 16,638             | 2,369              |
| Bank service charges                               |       | 60                 | 124                |
| Awareness  |       | 133                | 647                |
| Administration                                     |       | 231                | 94                 |
| Insurance  |       | 201                | 96                 |
| Accountant and examiner fees                       |       | 487                | 180                |
| Computer expenses                                  |       | 106                | 156                |
| Salary expenses                                    |       | 21,340             | 24,614             |
| <b>Total payments</b>                              |       | <b>60,567</b>      | <b>46,135</b>      |
| <b>Surplus (deficit) of receipts over payments</b> |       | <b>18,807</b>      | <b>(34,290)</b>    |
| Cash and bank balances brought forward             |       | 41,681             | 75,971             |
| <b>Cash and bank balances carried forward</b>      |       | <b>£60,488</b>     | <b>£41,681</b>     |

## BARTH SYNDROME UK

### Statement of Assets and Liabilities at 31 December 2023

#### Assets

The charity has the following bank and cash balances:

|                                   | <b>2023</b> | <b>2022</b> |
|-----------------------------------|-------------|-------------|
|                                   | <b>£</b>    | <b>£</b>    |
| Current account (CAF)             | 1,945       | 7,050       |
| Savings account (CAF)             | 28,543      | 4,631       |
| Restricted account (for reserves) | 30,000      | 30,000      |
|                                   | <hr/>       | <hr/>       |
|                                   | £60,488     | £41,681     |
|                                   | <hr/>       | <hr/>       |

#### Liabilities

None

Approved by the trustees on 07.03.2024 and signed on their behalf by Ralph Easterbrook



**Mr Ralph Easterbrook**  
**Chairperson**

## **INDEPENDENT EXAMINER'S REPORT TO THE TRUSTEES OF BARTH SYNDROME UK**

I report to the trustees on my examination of the accounts of Barth Syndrome UK (the Charity) for the year ended 31 December 2023.

### **Responsibilities and basis of report**

As the trustees of the Charity 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 Charity'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**

I have completed my examination. I confirm that no material 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 Trust as required by section 130 of the Act; or
2. the accounts do not accord with those records.

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.



Mr J P Foxwell FCCA FCIE  
independent-examiner.net

39 Enfield Road, Poole, BH15 3LJ

Date: 8 March 2024