



# **Barth Syndrome UK Annual Report 2021**

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## **BARTH SYNDROME UK**

### **TRUSTEES' REPORT**

The trustees present their report for the year ended 31 December 2021.

### **General Information**

**REGISTERED CHARITY NUMBER:** 1181830

**TRUSTEES:** Mrs Cheryl Parish – Chairperson  
Mr Geoffrey Parish – Treasurer  
Mr Stephen Cotterill  
Mr Ralph Easterbrook  
Mrs Isabel Easterbrook

**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.

## What is Barth Syndrome?

Barth syndrome is a rare, life-threatening, genetic mitochondrial disorder primarily affecting boys. 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, fatigue, and cardiolipin deficiency. In some people affected by Barth syndrome, the 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, Cheryl Parish:



2021 has proved to be as challenging for our families as 2020. As our community is highly vulnerable, we have had to maintain a higher degree of social distancing and consider very carefully how to balance safety and mental health and wellbeing. Our communication platforms have continued to provide possibilities for interaction, both for help and support alongside regular socialising opportunities. We did manage to host a small face to face get together, which for those able to attend, was highly rewarding. We fervently hope that 2022 will enable us to offer more occasions to be together in person. We were very pleased to be able to initiate a cognitive function study to assess the prevalence of any learning needs within the community, the results of which we intend to utilise in our future project planning. Money has also been allocated to continue funding a medical research program in partnership with our affiliate Barth Syndrome Foundation of Canada. Our volunteers, CEO and Trustees have worked tirelessly together to offer help and support to our families and to have effective dialogue around the world with our wider communities and scientists to give all Barth families a better future.

## MESSAGE FROM THE CHIEF EXECUTIVE, Michaela Damin:



2021 has been a bit of a mixed bag: on the one hand, we were relieved to see the resumption of some sort of normality but for our community, any social interaction has still been quite different compared to what it used to be.

We were excited to learn the results of our first ever clinical trial, but we were disappointed that the drug did not perform as well as we had hoped.

We had to navigate through the worrying decrease in fundraising events, but we were relieved that our families were staying safe and looking after themselves. And we recognised that many of us are exhausted with the ongoing pressures of work, home and health, exacerbated hundredfold during the ongoing pandemic.

We learned again the importance of quality mental health support while dealing with the crisis within our national health service which means that vital services are just not available to most of us.

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.

We are so grateful to each and every one who worked tirelessly, bravely and cheerfully through another challenging year.

All of us at Barth Syndrome UK pledge to keep working hard in 2022 to serve our affected families and make a positive difference in their lives.

Fond wishes  
Michaela

*Michaela Damin*

# REVIEW OF ACTIVITIES

## SUPPORTING FAMILIES IN 2021

### Private WhatsApp group

This group was set up at the start of the first lockdown and has been a very successful way for families to communicate with and support each other. It is owned by the members, rather than the charity and is open to all UK based individuals with Barth syndrome and immediate family members.

### One-to-One Support

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.

Our Chief Executive and our Family Services Support team are available to support new families through the testing process as well as provide ad hoc support to all member families.

### GDPR and Consents

In 2021 we refreshed all our families' consent forms to ensure that their choices around data sharing and storing are met, in line with GDPR legislation.

### Christmas Party

Barth families were finally able to meet up at our small Christmas party, which was held at the Star Inn, Bristol in December 2021. Private hire of a restaurant as well as social distancing and mask wearing meant that it was a COVID safe event for all. Party games, a delicious Christmas dinner and lots of fun was had by all.

### Bristol Barth Syndrome NHS Service

The Chief Executive worked closely with the Barth Syndrome Service to provide joined up care throughout 2021 in a tailored approach. This combined a mix of multidisciplinary virtual appointments, as well as the resumption of face-to-face clinics.

The charity provides financial assistance to affected families through a travel Costs Scheme aimed at making clinic attendance accessible for all throughout the UK.

The charity conducted an extensive service user survey towards the end of 2021 to identify areas of strengths and scope for improvement.

## **Transition: Older Boys' and Men Project**

The charity is in consultation with various experts and is continuing to identify needs and is planning possible projects to meet these needs. Examples include careers advice and support for independent living.

## **International Conference**

We, along with volunteers from Canada and the USA, have been planning the International Barth Syndrome Conference which is to be held in Florida in July 2022, Covid permitting.

## **SCIENCE AND MEDICINE**

### **Research Projects**

Barth Syndrome UK, along with Barth Foundation Canada, co-funded the following two research projects in 2021:

1. [Investigation of a new nutraceutical for treatment of Barth Syndrome](#)

**Robin Duncan**, PhD, Associate Professor, University of Waterloo, Waterloo, Ontario, Canada - \$41,580 over 2-year period

This project will assess the therapeutic potential and activity of a nutraceutical (a possible supplement therapy that is available without prescription) in preserving the viability of Barth syndrome cells.

Although COVID-19 resulted in delays due to the researchers not being able to access their laboratory for many months, this project is ongoing and will continue into 2022 when a second payment of approximately £10 000 will become due.

2. [Surveying TAZ genetic interactions and mutational landscape in human cells](#)

**Jason Moffat**, PhD, Professor, University of Toronto, \$50,000 over one year

This project enables us to better understand the TAFazzin gene and has the potential to expand our understanding about gene variants in our community. Data from this project could help us better understand the differences in severity of symptoms among our affected individuals.

The charity also plans to commit further funds towards new research projects in 2022.

### **Neurocognitive Service Evaluation Project**

Our work with our affected families raised a concern that there may be a higher rate of neurocognitive conditions like ADHD, autism, dyslexia and dyspraxia within our community. For this reason, the charity commissioned a review in 2021 of our UK families to systematically investigate this previously unknown aspect. Full results are expected in the first half of 2022.

With the knowledge that the National Health Service is unable to provide timely intervention for these kinds of issues, the charity expects to commit further funds in 2022/2023 towards helping families who need access to proper diagnosis and support for any neurocognitive condition.



## **Cardioman Clinical trial**

CARDIOMAN was the UK's first ever clinical trial using a repurposed medication called bezafibrate, which is normally used for treating lipid disorders.

This clinical trial was spearheaded by Professor Colin Steward – and later by Dr Guido Pieleles – and was sponsored by the National Institute for Health Research (NIHR).

The affected individuals who took part made regular trips to Bristol for intensive cardiac examinations, exercise testing and regular blood tests. To make the clinical trial more enjoyable, patients' visits were grouped so that they could spend some quality time together.

In 2021 we hosted an international online meeting for researchers to share the results of the trial. Although the results were not as positive as we had hoped, we learned many valuable lessons about conducting similar trials within our super rare community, lessons which we are keen to take forward to new clinical trial opportunities as they arise in the future. Our families demonstrated their incredible and ongoing commitment to taking part in innovative new research in an effort to find a treatment for our rare disease.

## **International Registry**

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

## **AWARENESS**

### **Website**

Work on our website has been ongoing in 2021, with a recent user commenting that it was 'very helpful! I've shared it with everyone who has questions about my pregnancy. It's very well organized and to the point.'

In 2021 we created a detailed set of resources around transitioning into Secondary School, details of which can be seen on our website.

## **FUNDRAISING**

We saw a devastating 50% reduction in fundraising events in 2021, indicative of the fatigue that COVID has caused, especially to our small group of committed Barth families, many of whom are understandably struggling. However, thanks to our committed volunteers and donors, we have managed to prevail through 2021. Our carefully managed reserves have left us in a stable position, and we are confident of being able to continue our ambitious and vital plans for the years ahead. Thank you to all our incredible fundraisers, donors and volunteers, you have been amazing as always.



## BARTH SYNDROME UK

### Receipts and Payments Account for the period 1<sup>ST</sup> January 2021 to 31 December 2021

	Notes	2021 Total £	2020 Total £
<b>RECEIPTS</b>			
Fundraising events		13,783	20,727
Gifts and donations		13,241	10,296
Gift Aid claims		3,855	2,271
Interest received		128	644
		<hr/>	<hr/>
<b>Total receipts</b>		31,007	33,938
		<hr/>	<hr/>
<b>PAYMENTS</b>			
Fundraising expenses		216	359
Science and medicine		22,177	5,720
Family support and education		1,439	966
Accountant		175	-
Bank service charges		182	86
Awareness		3	68
Subscriptions		76	-
Administration/Office		146	16
Insurance		96	
Computer expenses		196	243
Salary expenses		20,134	19,635
		<hr/>	<hr/>
<b>Total payments</b>		44,840	27,093
		<hr/>	<hr/>
<b>Surplus (deficit) of receipts over payments</b>		<b>(13,833)</b>	<b>6,845</b>
Balance brought forward at 1 January 2021		89,990	83,145
		<hr/>	<hr/>
		<b>£76,157</b>	<b>£89,990</b>
		<hr/> <hr/>	<hr/> <hr/>

## BARTH SYNDROME UK

### Statement of Assets and Liabilities at 31 December 2021

#### Assets

The charity has the following bank and cash balances:

	<b>2021</b>	<b>2020</b>
	<b>£</b>	<b>£</b>
Current account (CAF)	2,095	1,942
Savings account (CAF)	24,062	38,048
Restricted account (for reserves)	50,000	50,000
	<hr/>	<hr/>
	<b>£76,157</b>	<b>£89,990</b>
	<hr/>	<hr/>

#### Liabilities

None

Approved by the trustees on 4 February 2022  
and signed on their behalf by



**Mrs Cheryl Parish**  
**Chairperson**

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

I report to the trustees on my examination of the accounts of the charity for the year ending 31 December 2021.

### **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 charity 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 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.



**Jason Foxwell FCCA FCIE**  
independent-examiner.net

39 Enfield Road, Poole, BH15 3LJ

Date: 7 February 2022