



SPRING 2019

A New Name for 2019...



Board of Trustees: Cheryl Parish (Chair) Ralph Easterbrook, Helen Coleman (Secretary), Geoff Parish (Treasurer), Isabel Easterbrook with newly appointed Chief Executive, Michaela Damin at a recent meeting in Hampshire

Barth Syndrome Trust Changes Name to Barth Syndrome UK

A new name but our Vision and Mission remain unchanged.

The Barth Syndrome Trust was originally set up under a Trust Deed. This is quite an old fashioned type of structure and leaves the trustees exposed as there is no limited liability. In 2017 we started to think about changing our structure in order to protect the existing trustees and to make it easier to recruit new trustees in the future.



From January 2019, Barth Syndrome UK has been created as a Charitable Incorporated Organisation (CIO).

The main intended benefits of the new entity are that it has legal personality, the ability to conduct business in its own name to hire employees, and limited liability so that its members and trustees will not have to contribute in the event of financial loss.

Michaela Damin hired as part time Chief Executive



It is with great excitement that we announce that we have hired Michaela Damin as our Chief Executive.

Michaela has been tireless in her volunteer work for the charity and is dedicated to improving the lives of those affected by Barth syndrome. She brings years of experience of managing the charity and she has a son with the condition.

We are excited about growing the organisation under her leadership and we are actively looking for volunteers to help us.

CARDIOMAN UK Clinical Trial



Mitchell (above) and Alejandro (below) take part in CARDIOMAN



The **CARDIOMAN** trial, the first clinical trial in the UK to test an interventional therapy in Barth syndrome, enrolled the first patient in March 2019! The trial is sponsored by the National Institute for Health Research (NIHR) to test bezafibrate, a lipid-lowering drug that has been safely used to treat hypercholesterolemia since 1978.

CARDIOMAN aims to investigate the effectiveness of bezafibrate on lipid metabolism and subsequent heart function in boys and young men with Barth syndrome. Peak oxygen consumption, as well as heart function of participants during exercise stress, will be measured by cardiac ultrasound imaging.

Michaela Damin, Barth Syndrome UK's visionary founder, welcomed this historic and hopeful event, the culmination of many years of hard work and thanked the researchers and families involved saying *"We firmly believe that clinical trials in rare diseases such as Barth syndrome require cross-sector partnership."*

All participants receive 4 months of bezafibrate and 4 months of the placebo, the order of which is randomly determined. Both arms will have a 1-month washout period between the intervention and placebo, where no treatment is given. Results are expected by the end of 2019.

We have the cause. Do YOU have the effect? This month's focus is on... Social Media

We are a small charity run solely on donations of time and funds from people who have a connection to Barth syndrome. Do you know someone who has experience in **social media**? If so, we would appreciate their help!

Have you seen some of our latest resources? Find them on our website

University Hospitals Bristol  NHS Foundation Trust

[Understanding Feeding in Barth Syndrome](#)
[A Health Professionals Guide to the Early Years](#)



Written by Nicol Clayton

Specialist Paediatric Dietitian, NHS Specialised Services Barth Syndrome Service, Bristol Royal Hospital for Children

With contributions from:

Professor Colin Steward and Dr Germaine Pierre, NHS Specialised Services Barth Syndrome Service, Bristol Royal Hospital for Children

Dr Richard Kelley, Former Professor of Paediatrics, John Hopkins University School of Medicine, Baltimore

Dr Stacey Reynolds, Associate Professor in Occupational Therapy, Virginia Commonwealth University

Michaela Damin, Founder and Chair of the UK Barth Syndrome Trust.

The families of children with Barth syndrome



Barth Syndrome Education Guide

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Last Reviewed: March 2019