



Annual Report and Financial Statements

To the year ended
31 December 2023

myotubular trust
FINDING STRENGTH



UK Registered Charity, 1137177
A Company Limited By Guarantee

TRUSTEES' ANNUAL REPORT 31 December 2023

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The Trustees of the Myotubular Trust take pleasure in presenting their annual report and the financial statements for the year ended 31 December 2023.

Objects and Activities

The objects of the Myotubular Trust as agreed by the Charity Commission are;

- the relief of disability and the extension of life for babies, children and adults suffering from myotubular myopathy
- by promoting the study of, and research into, the treatment and cure of the muscle weakness caused by myotubular myopathy.

The Trust focuses on five outcomes to enable the achievement of these objectives:

1. Bringing families together
2. Promoting and funding international research
3. Sharing knowledge
4. Promoting innovative thinking and collaboration
5. Working with our extended families.

We bring about the public benefit required of all charities, by the activities of:

- making grants to organisations and individuals
- providing support, advice, and information
- sponsoring research.

Myotubular Myopathy and Centronuclear Myopathy

Myotubular myopathy is a rare and extremely life-threatening muscle condition, which causes profound muscle weakness, usually from birth. It is one of a group of conditions called **centronuclear** myopathies – under a microscope the nucleus of the muscle cell of someone affected is in the **centre**, rather than at the usual edge of the cell.

These conditions are genetic in inheritance. The gene mutation for x-linked myotubular myopathy is carried on the x chromosome (xlmtm), and is the most common form, affecting mainly boys. They usually do not survive their babyhood – the statistics have been under review in the last few years and the current view is that 50-75% die in their first year, and very few make it to adulthood. Even rarer genetic forms, either dominant or recessive in inheritance, can affect both males and females and are generally milder in severity but not always. They usually emerge in adulthood, but again it is not uncommon for children to be affected by these other forms. The terms myotubular and centronuclear myopathy both describe 'our' condition.

Children and adults with myotubular myopathy usually require vigilant nursing care, ventilators to breathe, power wheelchairs to get around, and are often fed via a tube into the stomach. Carrier females, and those affected by some of the rarer forms which can manifest in adult life, may develop muscle weakness and problems with eating and drinking; walking and movement; muscle fatigue and susceptibility to respiratory weakness and associated infections.

While myotubular myopathy has historically been considered primarily a muscle disease, and care has focused on these respiratory challenges and complications, the last four years have shone a light on the possible liver complications that some patients may suffer from. In the first two clinical trials for XLMTM and CNM, adverse events were reported in liver function tests, and tragically there were four deaths related to the liver. A new push in our field will be needed, to understand how the liver is impacted and what that means for future clinical trials, and just as importantly for day-to-day care of all patients.

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2023 – where are we now

In the 18 years since the Myotubular Trust was founded, the landscape has changed markedly. Day to day management of the condition has improved with evolving respiratory support techniques and, wonderfully, many children are living longer. Hence the change in survival data – something to be celebrated. Substantial progress has been made in understanding myotubular and centronuclear myopathy in those 18 years, and research into a treatment, or cure, has made great steps forward, including the first human clinical trials.

However, there remain many fundamental questions about the condition, and why and how these genetic variations, and the proteins they impact, cause muscle weakness. There have been heartbreaking setbacks in the clinical trials - the gene therapy trial is still on hold after the death of four participating patients, and the antisense trial sponsored by the biotech start-up, Dynacure, was terminated in 2022 due to concerning liver test results. Combined with the history of some of those with XLMTM (but not all) suffering from liver complications over the years, we now urgently need to uncover the true natural history of the liver, and the mechanism of action by which a lack of myotubularin is implicated in the liver.

The Trust was established in 2006 by two parents of boys with myotubular myopathy, simply to raise research funds. There was no other way for passionate parents to ring-fence money to fund research into a treatment or cure for this condition, even though we did at the time approach some larger charities for whom we had hoped to fundraise. We also realised very quickly that it was essential to support the profile of the condition within the neuromuscular research community – to compete for brains and innovative ideas – and to support those living with, and bereaved by, myotubular myopathy.

There are new understandings of the condition emerging particularly from the clinical trials, which present several new challenges, but also new avenues of research. We need to be here to voice the urgency of this work, and to be a source of funding too. We can be fleet of foot as an organisation when a fundamental new question emerges, as we were when we changed our grant calls to include questions about the liver. There was such hope when the first human clinical trials began for myotubular and centronuclear myopathy, and the difficult years that have ensued since then have shown us how important it continues to be to keep these conditions at the forefront of groundbreaking research.

We are dedicated to continuing to make a meaningful difference - there is clearly a vital role for a disease specific research funder in the world of rare disease, along with a tangible need to support patients and families. We are conscious though of 'Founder syndrome' and the voice of the living patient. We aim to take every opportunity to invite in other patients, parents and families to take key roles and become leading voices in this work. We recognise that no one organisation can provide all the answers, and it is our ambition in the next 5 years to support the initiatives of others, particularly our younger community.

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Since founding in 2006...we have:

1. Raised over **£2.9M**, through family-led fundraising, networks and relationships.
2. Made **18 research grants** to researchers in world class laboratories, with the support of a highly regarded Scientific Advisory Board.
3. Funded the proof of principle project for gene therapy that led to the **first ever clinical trial** for myotubular myopathy.
4. Funded **other proof of principle projects** that progressed to clinical trial.
5. Funded research grants that contributed to the discovery of **two new genes** implicated in centronuclear and myotubular myopathy.
6. Hosted **five family conferences**, bringing together affected individuals and families, researchers, clinicians, and other health professionals, and arranged several family meetings by **Zoom both during the pandemic and beyond** with presentations from, and discussions with, global experts and scientists.
7. Created the first disease specific international **Patient Registry** to gather vital information on the condition and funded its migration to the stability and credibility of a university setting.
8. Participated in a European Neuromuscular Centre workshop on Mouthpiece Ventilation, resulting in the publication of **MPV international best practice guidelines**.
9. Been invited by **Great Ormond Street Hospital Children's Charity** to be one of their small number of rare disease charity partners in their annual national funding call focusing on complex or rare childhood diseases.
10. Sponsored a **Standards of Care** meeting for clinicians, patients, and their healthcare professionals at the Royal Brompton Hospital.
11. Presented for LifeArc on **drug repurposing for rare diseases** and contributed to their published expert advice.
12. Contributed to the first ever **Congenital Myopathies Masterclass** for European healthcare providers. The Masterclass was organised by TREAT-NMD and included sessions led by world leading experts on myotubular and centronuclear myopathy.
13. Sponsored the UK focus group that contributed to the international study, and subsequent journal publication, **Lessons Learnt from Clinical Studies in Centronuclear Myopathies**.
14. Created a great band of **committed supporters**, raising funds for us year on year.
15. Developed strategic relationships with **key stakeholders** in the fields of neuromuscular disease, rare disease, UK regulatory bodies, and highly regarded UK children's charities including the UK umbrella muscle disease charity, MDUK.
16. And *most importantly*, brought together **families** affected by myotubular and centronuclear myopathy, who support the Trust's fundraising and each other's lives.

In 2023 specifically...we have:

1. Raised £117,500.
2. Continued to substantially add to our Family Factsheets, the purpose of which is to share personal family case studies to support other families in their care and decision making.
3. Invited applications for a joint grant round in partnership with Great Ormond Street Children's Charity.
4. Awarded a £300,000 grant to Dr James Dowling at Sick Kids, Canada to study liver disease in X-linked myotubular myopathy, in partnership with the UK muscle disease charity MDUK.
5. Continued our collaboration with the US patient organisation, MTM-CNM Family Connection, to run the MTM-CNM Liver Collaborative Working Group, bringing together a range of leading global experts, scientists, experienced clinicians, and biotechs, to fast-track cooperation on the understanding of liver issues emerging from current clinical trials.
6. Worked with the Myotubular and Centronuclear Myopathy Patient Registry, through the Liver Collaborative, to develop, launch and promote a new section of academic, clinical

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- and patient curated questions about liver health, leading to 121 patients sharing their liver data within 4 months.
7. Contributed to an academic presentation based on this data at the World Muscle Society meeting in October 2023.
 8. Co-hosted a webinar for patients and families to hear from worldwide experts on liver issues and recommendations for clinical care.
 9. Set up a research team to investigate possible muscle symptoms and lived experience of xlmtm carrier women in the UK and Ireland and disseminated a survey to carrier women in UK and Ireland which was resoundingly positively received.

Every single year since founding in 2006 we have raised 100% of our running costs separately from the funds raised by families and supporters, meaning that once again in 2023 we met our ambition to channel all the funds raised by supporters into research.

Family Support in 2023

Advocacy and Family Support

Specific support is needed for myotubular and centronuclear myopathy patients and families, mainly because of the rarity of centronuclear and myotubular myopathy. Usually, the child's or patient's GP or doctor will never have come across the condition before - making being diagnosed a frightening and bewildering experience for all concerned. Being hurled suddenly, and usually without warning, into a world of medical jargon; high level nursing; complex healthcare; and social care systems, families and individuals are grateful to know they are not alone, and that we, and others like them, are willing to share the burden of responsibility by helping them understand their experiences and to navigate the systems.

At a time when parents are supposed to be celebrating the birth of an infant, our families are often instead in the position of having to make important choices around giving their child the best quality of life, and a chance of longer-term survival. The information these families are given by their hospital care teams to make those decisions is sometimes inaccurate, often patchy, and even delivered by someone who has never seen a child with the condition before. When a baby is finally stable enough to go home, it is not unusual for the transition process from hospital to home to take 1 year to 18 months.

The need for support/information caused by a lack of understanding of the impacts of such a rare, devastatingly life threatening condition re-emerges many times throughout a patient's life - and again at times of change, such as when there is a physical change (such as losing the ability to walk or developing scoliosis), during periods of ill health, when moving home or into a new phase of education or work, around family planning, or practical day-to-day living issues.

In 2023, while providing individual support for individual issues for families, we also continued to add to **Factsheets**, our substantial repository of publicly available patient case studies and experiences to be shared. We are very grateful to all the patients and families who have contributed to these case studies. They are invaluable.

We also in 2023 continued to advocate for more knowledge to be collated and shared about liver health in myotubular myopathy. As part of the Liver Collaborative Working Group, we advocated for and supported the development of novel guidance on ongoing monitoring, and care for liver health. This '**One Page Liver Information Sheet**' was truly an international initiative and included both muscle and liver contributors from King's College London; Harvard Medical School; Radboud University, The Netherlands; the Hospital for Sick Kids, Toronto; and representatives from the medical teams of the companies who carried out the clinical trials in myotubular and centronuclear myopathy.

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We also arranged an international education and communication webinar on lessons learnt so far about liver issues and preparing the patient community for the need to gather data on the liver. This webinar was organised in partnership with the US patient advocacy organisation, MTM-CNM Family Connection.

The international study, ***Lessons Learnt from Clinical Studies in Centronuclear Myopathies***, led by Radboud University, the Netherlands, and in which the Myotubular Trust participated, resulted in an academic publication in 2023. The conclusion of the study emphasised the importance of involving patients, caregivers, and patient communities in the design, development, and evaluation of clinical studies and trials. We, and the whole research project team, hope this will provide a valuable resource for those sponsoring clinical trials in the future, and most importantly for patients and their families considering taking part in a clinical trial.

The global closed Facebook group managed by Myotubular Trust – ***Myotubular Myopathy*** - continues to go from strength to strength, with membership reaching 900 members in 2023. This group continues to provide real-time, real-life advice and perspectives for families who regularly tell us they'd be very alone without this community.

Research in 2023

The Myotubular Trust was set up to raise funds to leverage research findings being made, and techniques being trialed, in 'more common' muscle diseases. The ultimate goal of the Trust is to invest in a diverse number of routes to a cure for all genetic forms of myotubular and centronuclear myopathy, or in treatments that alleviate the most severe symptoms. We do this by investing in the very best translational research, peer reviewed to the highest international standards, supported by an eminent Scientific Advisory Board.

Current Grants

Tamoxifen clinical trial

This grant, which is co-funded with Great Ormond Street Hospital Children's Charity, is for the UK arm of a clinical trial to test how the oncology drug, Tamoxifen, works in improving motor and respiratory function in patients with myotubular myopathy. The trial is being led by Dr Giovanni Baranello, and Professor Francesco Muntoni, at the UCL Great Ormond Street Institute of Child Health. In 2023 it completed the recruitment of children in the UK, aged 2 and above, who are affected by x-linked myotubular myopathy, and is part of the global drug repurposing trial that resulted from the work the Myotubular Trust funded with Dr Jim Dowling at Sick Kids Canada. The global trial is led by Dr Dowling and is a double-blind cross over trial - involving 6 months on either a placebo or the drug, with a three month 'wash out period' in between.

Next generation gene therapy

Dr Ana Buj Bello was the recipient of the first ever research grant from the Myotubular Trust in 2008 for her pioneering work on gene replacement therapy for xlmtm. The goal of this latest 2022 grant to Dr Buj Bello and her team at Genethon is to develop an even more specific and potentially safer 'vector' to carry the missing protein, myotubularin, which they hope will ultimately be brought to clinical trial. They also aim to better characterise the liver in laboratory tests, both before and after gene therapy. Based on this understanding and new developments in the technology of AAV vectors, they aim to generate optimised vectors with increased potency and specificity for MTM1 gene delivery.

Studying liver disease in X-linked myotubular myopathy

Dr Jim Dowling has been the recipient of a number of research grants from the Myotubular Trust. The purpose of this 2023 grant to Dr Dowling and his team at Sick Kids is to better understand the cause of liver disease in people living with XLMTM, and to help develop a treatment. It has become clear in recent years that some individuals with X-linked myotubular myopathy can have problems with their liver. Liver complications have also had an impact on

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the progress of clinical trials. Dr Dowling's work will generate new information on how the liver works in those with XLMTM. This grant was made in partnership with the UK muscle disease charity, MDUK, who are contributing £60,000 over 3 years to the overall grant of £300,000. We are delighted to be partnering with MDUK on this important grant, and believe it is testament to how vital it will be for myotubular myopathy, and likely many other neuromuscular conditions, to better understand the impact of gene therapy, and other treatments, on the liver in clinical trials.

Clinical trial update

Gene therapy

Virus mediated gene therapy received funding from the Myotubular Trust as far back as our first grant award in 2009 to Dr Ana Buj Bello at Genethon, Paris. Dr Buj Bello established proof of principle for this gene therapy, and with the success of her research funded by the Myotubular Trust (grants in 2009 and 2012), other funders subsequently joined in too.

Dosing patients in this trial began in 2018, and it is really important to state that there have been some remarkable outcomes for a number of patients. Ventilator independence has been achieved by boys previously on 24-hour ventilation and generally 'dramatic clinical improvements' were seen, as noted in the academic literature. Essentially, what has been proven is that gene therapy can reverse the severest of symptoms of myotubular myopathy.

Tragically though, 4 young patients died during the trial, and the trial remains on hold while the FDA and the sponsoring company investigate thoroughly. What has become very clear though is that there are aspects of the condition that we do not fully understand yet – including in relation to the liver.

We sincerely thank those affected by myotubular and centronuclear myopathy who took part in this trial and also the antisense trial sponsored by Dynacure that was halted in 2022. As one mother said "It is one thing to live without hope, it is quite another to have hope dashed." Clinical trials, particularly for conditions as severe as ours, require enormous commitment in terms of time, travel, logistics and patience. For the trial to end makes that commitment even harder to endure, and we thank them for that endurance, and for adding to the sum knowledge of our understanding of the condition.

The one clinical trial still ongoing in 2023 was the tamoxifen trial – TAM4MTM. Although the first funding for this trial was in place as early as 2019, setting it up had several setbacks, with Covid having a fundamental impact on timing. The first patients were dosed in Canada in 2021, but it proved more difficult to get it off the ground in the UK and the US. However, the excellent clinical trial team at Great Ormond Street Hospital finally recruited all UK patients in 2023 and began dosing and taking great care of those young boys.

On the road to all these clinical trials, the role of the Trust was to fund the proof of principle/validation work being proposed by so many outstanding researchers, paving the way for companies like Astellas Gene Therapies and Dynacure, or academic institutions such as Sick Kids Toronto and Great Ormond St. London, to take them to clinical trial. That funding makes a fundamental difference, and we are immensely grateful to all our supporters who have made this possible.

We are immensely grateful to our Scientific Advisory Board (SAB) for their time and their expertise and understanding to bring excellence and rigour to our funding decisions. The calibre of applications the Myotubular Trust attracts is due in no small part to the reputation of this group of scientists.

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The academic members of the SAB are:

- Professor Francesco Muntoni, Head of the Dubowitz Neuromuscular Centre, Great Ormond Street Hospital
- Dr Meriel McEntagart, Consultant Clinical Geneticist, St. George's Hospital, London
- Professor Michael Duchen, Professor of Physiology, University College London
- Dr Susan C Brown, previously Reader in Translational Medicine, Department of Veterinary Basic Science, Royal Veterinary College, University of London
- Professor Volker Straub, Harold Macmillan Professor of Medicine, Consultant in Neuromuscular Genetics and Paediatrics, Newcastle University
- Professor Dominic Wells, Professor in Translational Medicine, Royal Veterinary College

We are indebted for the guidance they gave to the Myotubular Trust throughout the year. We are also very grateful to the lay members of our SAB for their time. The lay members include parents, bereaved parents and patients.

Financial Review

Sustainable Fundraising – the Highlights of 2023

The post Covid fundraising landscape has changed substantially, and therefore the Trustees were very grateful that we were able to raise in the region of £120,000 once again this year. Although some challenge-type amateur sporting events have returned since Covid, the sponsorship environment has definitely changed – and people clearly find it harder to ask to be sponsored. This may be due to increasingly constrained personal finances during recent years. Despite that though, our families and their communities continue to find ways to raise funds for us – leveraging the 'ripple effect' from each child into an extended network of supporters has been so critical to our growth and stability.

2023 was the year of:

- The 17th Jack Blunsdon Golf Day
- A New Year Sea swim in the cold waters off southern Ireland
- Marathon runners in London, Belfast, Brighton and Dublin
- Half marathon runners in Oldham and Belfast
- Another fantastic Tough Mudder
- Birthday, christening and wedding donations

Sadly, 2023 was another year of many losses and we extend our gratitude to those who, in their time of grief, considered the Myotubular Trust for donations.

A special mention goes once again in 2023 to IGY Foundation who give us so much moral and financial support. Knowing how generous their philanthropy is, we fully realise there are many causes who look to them for help, and we are very grateful that they continue to value the work we do. Their support regularly helps us to be able to meet unexpected needs from research teams, and certainly helps with our running cost pledge.

We also received a very welcome grant of £21,000 from Astellas Pharmaceuticals to provide an educational grant to support the development of a patient leaflet; software development and translation of liver related questions on the patient registry; and educational materials and webinars.

We are also very grateful for the steady income from the supporters from whom we receive standing orders and Give as You Earn schemes during 2023. Thank you to you all.

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Fundraising Practice

The Trustees take their responsibilities to fundraising very seriously. To that end, we ensure that we adhere to the Fundraising Regulator's code of best practice and meet the Charity Commission's six principles of charity fundraising. For us, at our size, the areas of best practice we pay attention to are;

- providing information on how to fundraise safely,
- considered communication with all fundraisers, which is appropriate to our strategy,
- carrying out a proportionate process of due diligence around fund raising proposals,
- making a record of the issue and return of any charity collection materials,
- securing cash donations and banking them as soon as possible,
- not sharing personal data without explicit consent,
- including opt-out information on fundraising communications sent to a named individual,
- processing unsubscribe requests in a timely way,
- using funds as they have been directed by specific donors, in particular allocating restricted funds appropriately.

We adhere to the Data Protection Act 2018, General Data Protection Regulation (GDPR) and pay data protection fees to the Information Commissioner's Office (ICO).

Reserves Policy

The Trustees sole aim in expending cash reserves is to fund research into a cure or treatment for myotubular myopathy and they do not consider it appropriate to hold long-term reserves.

However, as each research project is granted, those funds will be held available to be released in staged payments, and new awards will only be made if the full cost of the relevant research project is available, and available to be ringfenced. This prudent financial policy is appropriate given our size and specialism.

This policy meant that instead of being hit by a cash flow crisis in 2020 and 2021, and the even worse possibility of not being able to pay for committed research grants, we were even able to announce new grant awards in 2020, announce another grant call for 2021 and prepare to announce another call in 2022 and 2023.

Plans for future periods

The Trust aims in 2024 to:

- Maintain excellence in family support, advocacy, information provision – in particular our Factsheets focus - and continue to make the introductions that families tell us are invaluable.
- Actively promote and support the Liver Questionnaire on the Myotubular and Centronuclear Myopathy Patient Registry to assist in the development of a new academic publication in time for World Muscle Society 2024 – in order to continue the interest in, and commitment to, understanding the liver issue in myotubular myopathy.
- Support the dissemination of liver related resources for patients to share with their medical teams, and liver data to share with the clinical and research communities.
- Promote, and fund, research that furthers our understanding of the liver and bile function in myotubular myopathy, with the goal of being able to impact both clinical trial planning and ongoing patient care.
- Share the results of TAM4MTM, the tamoxifen repurposing trial.

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- Continue to support the development of a UK carrier women study, building on the excellent work already being done in the Netherlands and Germany.
- Organise webinars on key topics of interest for the patient community and review the appetite for meeting in person once again.
- Continue to work with key stakeholders in the neuromuscular disease community, and strategic rare disease organisations.
- Continue to work with regulatory authorities on behalf of patients with myotubular and centronuclear myopathy.
- Plan ahead and prepare effectively for any future NICE review of treatments for myotubular and centronuclear myopathy, facilitating families' and patients' input.
- Continue to share information with, and promote collaboration between, other family and patient organisations, relevant scientific interest groups and research organisations.
- Continue to secure separate funding to cover the Trust's running costs, while giving our heart felt support all our family-linked fundraisers.

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Reference and administrative details of the Charity, its Trustees and advisors

The following were Trustees of the Trust and held office during the above period:

Patricia Allen
Gary Browning
Andrew Lennox
Anne Lennox
James Rosling

Trustees are appointed by the board of trustees and serve for three years (five years for Anne Lennox) after which period they may put themselves forward for re-appointment. The trustees meet three times per annum.

Office

Myotubular Trust
- charity registration number 1137177
- company registration number 07260229

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Independent Examiner

Michael Stone MA ACA
Jamieson Stone LLP
Windsor House
40/41 Great Castle Street
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W1W 8LU

Structure, Governance and Management

Constitution

The Myotubular Trust is an incorporated charity limited by guarantee and its governing document is the memorandum and articles of association dated 21 May 2010. The memorandum and articles of association has the same objects as that of the Trust Deed dated 26 January 2006 of the unincorporated charity. The Trust obtained charitable status under Section 4 of the Charities Act 1960 from the Charity Commission on 19 April 2006 under registration number 1113809 and following incorporation on the 20th May 2010 was registered with the Charity Commission with registration number 1137177, and with Companies House with company registration number 7260229.

Organisation and the Trustees

In selecting individuals for appointment, the trustees will have regard to the skills, knowledge and experience needed for the effective administration of the charity.

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Trustees' responsibilities statement

The Trustees (who are also directors of The Myotubular Trust for the purposes of company law) are responsible for preparing the Trustees' Report and the financial statements in accordance with applicable law and United Kingdom Accounting Standards.

Company law in England and Wales requires the Trustees to prepare financial statements for each financial year in accordance with United Kingdom Generally Accepted Accounting Practice (United Kingdom Accounting Standards and applicable law). The financial statements are required by law to give a true and fair view of the state of affairs of the charity and of the income and expenditure of the charity for that period. In preparing these financial statements the trustees are required to:

- Select suitable accounting policies and then apply them consistently
- Observe the methods and principles of the Charities SORP
- Make judgments and estimates that are reasonable and prudent
- State whether applicable UK accounting standards have been followed and statements of recommended practice, subject to any departures disclosed and explained in the financial statements and
- Prepare the financial statements on the going concern basis unless it is inappropriate to presume that the charity will continue to operate.

The trustees are responsible for keeping accounting records, which disclose with reasonable accuracy at any time the financial position of the charity and enable them to ensure that the financial statements comply with the Companies Act 2006. They are responsible for safeguarding the assets of the charity and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

Risk management

The trustees have examined the strategic, business and operational risks, which the charity faces and confirm that systems have been established to enable regular reports to be produced so that the necessary steps can be taken to lessen the risks.

INDEPENDENT EXAMINER'S REPORT FOR THE YEAR ENDED 31 DECEMBER 2023

Respective responsibilities of trustees and examiner

The trustees (who are also the directors of the company for the purpose of company law) are responsible for the preparation of the accounts. The charity's trustees consider that an audit is not required for this year under section 144 of the Charities Act 2011 (the Charities Act) and that an independent examination is needed.

Having been satisfied that the charity is not subject to audit under company law and is eligible for independent examination it is my responsibility to:

- examine the accounts under section 145 of the Charities Act,
- to follow the procedures laid down in the General Directions given by the Charity Commission (under section 145(5)(b) of the Charities Act, and
- to state whether particular matters have come to my attention.

Basis of independent examiner's statement

My examination was carried out in accordance with the General Directions given by the Charity Commission. An examination includes a review of the accounting records kept by the charity and a comparison of the accounts presented with those records. It also includes consideration of any unusual items or disclosures in the accounts, and the seeking of explanations from you as trustees concerning any such matters. The procedures undertaken do not provide all the evidence that would be required in an audit, and consequently, no opinion is given as to whether the accounts present a 'true and fair view' and the report is limited to those matters set out in the statement below.

Independent examiner's statement

In connection with my examination, no matter has come to my attention:

- 1) which gives me reasonable cause to believe that in, any material respect, the requirements:
 - to keep accounting records in accordance with section 130 of the Charities Act; and
 - to prepare accounts which accord with the accounting records and to comply with the accounting requirements of the Companies Act 2006 and with the methods and principles of the Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2019) have not been met; or
- 2) to which, in my opinion, attention should be drawn in order to enable a proper understanding of the accounts to be reached.

Michael Stone

Michael Stone MA ACA
Jamieson Stone LLP
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40/41 Great Castle Street
London
W1W 8LU

27 September 2024

STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2023

	Unrestricted Funds £	Designated Funds £	Restricted Funds £	Total Funds 2023 £	Total Funds 2022 £
<u>INCOMING RESOURCES</u>					
Donations and grants	42,788	41,000	-	83,788	107,499
Fundraising activities	30,535	-	-	30,535	38,525
Interest received	3,188	-	-	3,188	1,438
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Total incoming resources	76,511	41,000	-	117,511	147,462
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<u>RESOURCES EXPENDED</u>					
Research	-	16,151	-	16,151	40,704
Fundraising costs	(396)	-	-	(396)	-
Outreach work	14	-	-	14	-
Staff costs	49,961	-	-	49,961	49,961
Training	24	-	-	24	-
Accountancy & payroll	236	-	-	236	300
Independent examiner	1,000	-	-	1,000	1,000
Legal & professional	1,262	-	-	1,262	943
Trustee's indemnity	1,009	-	-	1,009	917
Travel costs	120	-	-	120	183
Printing & stationery	25	-	-	25	107
Postage & telephone	122	-	-	122	79
Subscriptions	85	-	-	85	252
Web & IT costs	1,112	-	-	1,112	1,658
Bank charges	109	-	-	109	236
Miscellaneous costs	42	-	-	42	319
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Total resources expended	54,725	16,151	-	70,876	97,944
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Net incoming/(outgoing) resources	21,786	24,849	-	46,635	49,518
Transfers between funds	(10,000)	10,000	-	-	-
	-----	-----	-----	-----	-----
Net movement in funds	11,786	34,849	-	46,635	49,518
	-----	-----	-----	-----	-----
Total funds brought forward	6,323	370,338	-	376,661	327,143
	-----	-----	-----	-----	-----
Total funds carried forward	18,109	405,187	-	423,296	376,661
	-----	-----	-----	-----	-----

All gains and losses arising in the year are included within the statement of financial activities

The accompanying notes form part of these financial statements

BALANCE SHEET AS AT 31 DECEMBER 2023

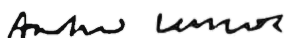
	£	2023 £	£	2022 £
CURRENT ASSETS				
Debtors and prepayments	41,958		32,030	
Cash at bank and in hand	387,268		348,676	
	-----		-----	
Total current assets	429,226		380,706	
	-----		-----	
CREDITORS				
Amounts falling due within one year	5,930		4,045	
	-----		-----	
Net current assets		423,296		376,661
		-----		-----
NET ASSETS		423,296		376,661
		-----		-----
FUNDS OF THE CHARITY				
Restricted funds		-		-
Unrestricted general funds		18,109		6,323
Unrestricted designated funds		405,187		370,338
		-----		-----
		423,296		376,661
		-----		-----

The company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 December 2023 and the members have not required the company to obtain an audit of its financial statements for the year ended 31 December 2023 in accordance with Section 476 of the Companies Act 2006.

The directors acknowledge their responsibilities for: (a) ensuring that the company keeps accounting records which comply with Sections 386 and 387 of the Companies Act 2006 and (b) preparing financial statements which give a true and fair view of the state of affairs of the company as at the end of each financial year and of its profit or loss for each financial year in accordance with the requirements of Sections 394 and 395 and which otherwise comply with the requirements of the Companies Act 2006 relating to financial statements, so far as applicable to the company.

The financial statements have been prepared in accordance with the provisions of Part 15 of the Companies Act 2006 relating to small companies.

The financial statements were approved by the trustees on 23 September 2024 and signed on their behalf:



.....
Andrew Lennox
Chairman/Trustee



.....
James Rosling
Treasurer/Trustee

The accompanying notes form part of these financial statements

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2023

1. BASIS OF PREPARATION

The financial statements have been prepared on the accruals basis of historic cost in accordance with the Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2019) and the Companies Act 2006 and the Charities Act 2011.

2. ACCOUNTING POLICIES

Incoming resources

Incoming resources are recognised when the charity becomes entitled to the resources, the trustees are virtually certain they will receive the resources, and the monetary value can be measured with sufficient reliability.

Where incoming resources have related expenditure (as with fundraising or contract income) the incoming resources and related expenditure are reported gross in the Statement of Financial Activities.

Grants and donations are recognised when the charity has unconditional entitlement to the resources.

Incoming resources from tax reclaims are recognised at the same time as the gift to which they relate.

Contractual income and performance related grants are recognized once the related goods or services have been delivered.

Investment income is included in the accounts when receivable.

Resources expended

Resources expended are recognised as soon as there is legal or constructive obligation committing the charity to pay out resources.

Resources expended include attributable VAT which cannot be recovered.

Funds

Restricted funds are funds to be used for specific purposes as declared by the donor.

Designated funds are monies set aside from general funds and designated for specific research awards by the trustees.

Unrestricted funds are donations and all other incoming resources without a specified purpose and which are available as general funds.

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED)

FOR THE YEAR ENDED 31 DECEMBER 2023

3. TAXATION

The Myotubular Trust is a registered charity and is not liable to Income Tax or Corporation Tax on income derived from its charitable activities.

4. RESOURCES EXPENDED

	2023 £	2022 £
Research	16,151	40,704
Independent examiner's remuneration	1,000	1,000
	-----	-----

The principal research grants awarded during the year were as follows:

	2023 £	2022 £
Hnia at Inserm	9,958	38,929
Inserm(Buj Bello) 2023-25	4,167	-
Newcastle University re Treat NMD	2,026	-
Research Consultancy	-	1,775
	-----	-----

5. DEBTORS AND PREPAYMENTS

	2023 £	2022 £
Gift Aid	458	1,530
Prepayments and accrued income	41,500	30,500
	-----	-----
	41,958	32,030
	-----	-----

6. CREDITORS FALLING DUE WITHIN ONE YEAR

	2023 £	2022 £
Accruals	5,930	4,045
	-----	-----

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 DECEMBER 2023

7. RELATED PARTY TRANSACTIONS

There were no related party transactions by the trustees for the year (2022: None).

One Trustee received remuneration of £20,000 during the year ending 31 December 2023 (2022: £20,000).

The trustee's remuneration and other administration costs are funded from unrestricted corporate and charitable donations, which were specifically raised for that purpose.