



Annual Report and Financial Statements

To the year ended
31 December 2024

myotubular trust
FINDING STRENGTH



UK Registered Charity, 1137177
A Company Limited By Guarantee



TRUSTEES' ANNUAL REPORT 31 December 2024

TRUSTEES' ANNUAL REPORT 31 December 2024

The Trustees of the Myotubular Trust take pleasure in presenting their annual report and the financial statements for the year ended 31 December 2024.

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.

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.

The Myotubular Trust's primary objective is to facilitate and fund medical research that advances treatments and, ultimately, help to find a cure for myotubular and centronuclear myopathy. Alongside this, the Trust provides support and information to families affected by the condition and works to enhance awareness among healthcare professionals, industry and the public.

To achieve these aims, the Trust raises funds through a combination of individual donations, corporate partnerships, grants from other Trusts and Foundations, and community-led fundraising initiatives. All activities are designed to ensure the best possible use of available resources, maximising impact for the community we serve.

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 possible 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. It can even be fatal for a small number of seriously affected carrier women.

While myotubular myopathy has historically been considered primarily a muscle disease, and care has focused on the respiratory challenges and complications, the last five years have shone a light on liver complications too. In all three of the clinical trials myotubular myopathy (XLMTM) and centronuclear myopathy (CNM), adverse events were reported in liver

TRUSTEES' ANNUAL REPORT 31 December 2024

function tests, and all three were prematurely halted for this reason. Tragically, four boys died in the gene therapy trial. A new push in our field is underway, 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.

2024 – where are we now

In the 19 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. The change in survival data is truly something to be celebrated. Substantial progress has been made in understanding myotubular and centronuclear myopathy in those 19 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; the 'antisense' trial sponsored by the biotech start-up, Dynacure, was terminated in 2022 due to concerning liver test results; and finally, the last of the three clinical trials so far for myotubular myopathy - the repurposing of tamoxifen - had to be halted in 2024 due to serious liver adverse events. Combined with the history of some of those with XLMTM (but not all) suffering from liver complications over the years, the community now urgently needs 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 such hope when, only 10 years later, 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.

Since founding in 2006...we have:

1. Raised over **£3M**, through family-led fundraising, networks and relationships.
2. Made **19 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 the only other two human clinical trials for our condition.
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.

TRUSTEES' ANNUAL REPORT 31 December 2024

8. Participated in a European Neuromuscular Centre workshop on Mouthpiece Ventilation, resulting in the publication of **MPV international best practice guidelines**.
9. Been invited on an ongoing basis 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, and UK regulatory bodies
16. We co-fund research projects with highly regarded UK charities - Great Ormond Street Hospital's Charity, and the UK umbrella muscle disease charity, MDUK.
17. And *most importantly*, brought together **families** affected by myotubular and centronuclear myopathy, who support the Trust's fundraising and each other's lives.

In 2024 specifically we have:

1. Raised £138,416.
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 which resulted in a grant to a team at UCL for a research project investigating the liver in XLMTM.
4. 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.
5. Worked with the Myotubular and Centronuclear Myopathy Patient Registry, through the Liver Collaborative, to continue to communicate the value of a new section of academic, clinical and patient curated questions about liver health, leading to 219 patients sharing their liver data by the end of 2024 – a remarkable number for a 'new' topic for a rare disease.
6. Worked with the Registry and the Liver Collaborative to add a new set of Nutrition questions to the registry.
7. Organised a webinar for patients and families to hear directly from the research teams about the implications of the tamoxifen clinical trial being halted.
8. Continued to project manage a research team to investigate possible muscle symptoms and lived experience of xlmtm carrier women in the UK and Ireland.

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 2024 we met our ambition to channel all the funds raised by supporters into research.

TRUSTEES' ANNUAL REPORT 31 December 2024

Family Support in 2024

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 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 how 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 can be patchy and possibly 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 particularly 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 2024 we have supported families who were:

- Applying for re-housing and needing to communicate the scale of equipment and storage needed for a growing young man with this myotubular myopathy
- Making decisions about palliative care at the point of diagnosis in a neonatal setting
- Seeking advice on the most appropriate specialist equipment for their child
- Requesting introductions to others with their own mutation and in their own age group
- Enquiring about the current status of clinical trials and future potential research
- Wanting to discuss a child's mental health at a point on life when they become aware that they are 'different' to their peers

In 2024, 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 that are shared in order to help improve the standards of care and quality of life of our community. We are very grateful to all the patients and families who have contributed to these case studies. They are invaluable.

The global closed Facebook group managed by Myotubular Trust – **Myotubular Myopathy** - continues to go from strength to strength, with membership reaching 950 members by the end of 2024. 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.

TRUSTEES' ANNUAL REPORT 31 December 2024

Research in 2024

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.

The Myotubular Trust was very gratified to have provided proof of principle funding for all three of the clinical trials that came to clinic in the last 8 years.

Sadly, in 2024, the TAM4MTM clinical trial, which investigated the repurposing of tamoxifen for treating XLMTM, was discontinued due to observed impacts on liver function. This brought to an end all three of the first clinical trials for myotubular and centronuclear myopathy, as in all of them, adverse events were reported in liver function tests. Following this development, in December 2024 the Myotubular Trust hosted a webinar for the community to discuss the trial's outcomes and implications for future research.

We sincerely thank those affected by myotubular and centronuclear myopathy who took part in the trials. 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 trials 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.

It is very clear that for research into a treatment for myotubular and centronuclear myopathy to progress, we need to understand more about how the liver is affected, and what goes on when there are liver complications.

Current grants

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 our current 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, to muscle cells. An approach 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 our current 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. This grant was made in partnership with the UK muscle disease charity, MDUK. 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.

TRUSTEES' ANNUAL REPORT 31 December 2024

An Advanced Multi-Organoid Platform for Therapy Modelling in X-Linked Myotubular Myopathy

In 2024, as part of their annual grant call for rare paediatric disease, we partnered with Great Ormond Street Hospital Children's Charity in a three-year grant of £248,940 to Professor Francesco Saverio Tedesco and Dr. Valentina Maria Lionello at University College London. This project focuses on creating patient-specific 3D 'mini-muscles and livers' using human induced pluripotent stem cells reprogrammed from individuals with X-linked myotubular myopathy (XLMTM). These models aim to enhance our understanding of the disease and serve as platforms for testing potential gene therapies.

These liver focused grants underscore our commitment to funding and facilitating research that enhances understanding and that paves the way for effective treatments for myotubular and centronuclear myopathy.

We are immensely grateful to our Scientific Advisory Board (SAB) for their time and their expertise and understanding to bring excellence and rigor 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.

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, previously 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.

TRUSTEES' ANNUAL REPORT 31 December 2024

Financial Review

Achievements and Performance in 2024

In 2024 we raised £138,416 and have continued to drive forward our mission, funding key research projects, forging partnerships and growing our support network. This year, we were fortunate to receive generous grants from IGY Foundation*, Astellas Pharmaceuticals**, Global's Make Some Noise, BBC Children in Need, and Jeans for Genes. These funds played a crucial role in sustaining and expanding our work, allowing us to continue supporting cutting-edge research and vital family support initiatives. Their support means that, yet again, we have been able to commit 100% of the funds raised by families, their friends and colleagues, to research. This has been so important to us as a principle, ever since founding, and it is a source of great satisfaction that we have achieved this '100% pledge' every year for 19 years.

*A special mention goes once again to IGY Foundation who have given us so much moral and financial support over the years. 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 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 £25,000 from Astellas Pharmaceuticals to support the ongoing development of the patient registry, to support for the coordination of a study of xlmtm carrier women in the UK, and to assist with the costs of organising our Hope Walks. This grant constitutes less than 18% of our 2024 income.

Community and Fundraising Activities

Our dedicated community of supporters has played an essential role in helping us raise both awareness and funds in 2024. Trust-led events included:

- The London Hope Walk, which saw over 90 walkers take to the streets and, as ever, every single one of them joined in recognition of someone with the condition. This was the 17th year of the London Hope Walk and this event over those years has raised over £250,000 – so far!
- The first-ever Manchester Hope Walk, where 70 walkers participated, representing 5 families of boys with myotubular myopathy
- Ongoing engagement with Easyfundraising – with a regularly growing number of supporters
- The growth of our regular giving programme, with new donors joining in 2024, ensuring sustained support for our work.

TRUSTEES' ANNUAL REPORT 31 December 2024

In addition to Trust-led initiatives, families and supporters - across the world - undertook their own fundraising activities, demonstrating incredible commitment and creativity. Some of the inspiring events included:

- A half marathon run to raise funds, and awareness
- A surfing challenge, showcasing both endurance and spirit
- A Tough Mudder event
- A skydive in Australia in memory of a beloved brother
- Another successful Jack Blunsdon Golf Tournament – the 18th - bringing together supporters yet again for a day of fundraising on the course in memory of Jack
- A 60-mile bike ride by a devoted grandad in memory of his grandson, highlighting the personal connections that drive our supporters
- A boxing/wrestling event at the Bridge Centre, in remembrance of a special little boy
- A school fundraiser, engaging the next generation in charitable giving and awareness efforts.

We were also very grateful to the fundraisers in 2024 who applied to their company's matched funding schemes. These invaluable schemes that financially match their employees' efforts for charities are a fantastic source of funding for us.

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 2024. Thank you to you all.

TRUSTEES' ANNUAL REPORT 31 December 2024

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.

Plans for future periods

The Trust aims in 2025 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.
- Organise and support Hope Walks in London and in at least one other location in the UK or Ireland.
- Actively promote and support the Liver Questionnaire and Nutrition Questionnaires on the Myotubular and Centronuclear Myopathy Patient Registry, to assist in the development of a new academic publication in time for World Muscle Society 2025.
- 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.
- 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 investigate the interest in meeting in person once again.

TRUSTEES' ANNUAL REPORT 31 December 2024

- 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 for running costs to uphold our pledge of investing 100% of funds raised by families and their networks directly to research.

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

15a Barnard Road
London SW11 1QT

Bankers

CAF Bank Limited
Kings Hill
West Mailing
Kent
ME19 4JG

The Co-operative Bank
PO Box 250
Skelmersdale
WN8 6WT

Independent Examiner

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

TRUSTEES' ANNUAL REPORT 31 December 2024

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.

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 2024

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
Windsor House
40/41 Great Castle Street
London
W1W 8LU

17 September 2025

STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2024

	Unrestricted Funds £	Designated Funds £	Restricted Funds £	Total Funds 2024 £	Total Funds 2023 £
<u>INCOMING RESOURCES</u>					
Donations and grants	53,230	52,500	-	105,730	83,788
Fundraising activities	27,533	-	-	27,533	30,535
Interest received	5,153	-	-	5,153	3,188
Total incoming resources	85,916	52,500	-	138,416	117,511
<u>RESOURCES EXPENDED</u>					
Research	-	89,453	-	89,453	16,151
Fundraising costs	2,438	-	-	2,438	(396)
Outreach work	-	-	-	-	14
Staff costs	49,961	-	-	49,961	49,961
Training	-	103	-	103	24
Accountancy & payroll	366	-	-	366	236
Independent examiner	1,000	-	-	1,000	1,000
Legal & professional	92	-	-	92	1,262
Trustee's indemnity	1,042	-	-	1,042	1,009
Travel costs	954	-	-	954	120
Printing & stationery	-	-	-	-	25
Postage & telephone	105	-	-	105	122
Subscriptions	205	-	-	205	85
Web & IT costs	2,380	-	-	2,380	1,112
Bank charges	210	-	-	210	109
Miscellaneous costs	-	-	-	-	42
Total resources expended	58,753	89,556	-	148,309	70,876
Net incoming/(outgoing) resources	27,163	(37,056)	-	(9,893)	46,635
Transfers between funds	(37,056)	37,056	-	-	-
Net movement in funds	(9,893)	-	-	(9,893)	46,635
Total funds brought forward	18,109	405,187	-	423,296	376,661
Total funds carried forward	8,216	405,187	-	413,403	423,296

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 2024

	£	2024 £	£	2023 £
CURRENT ASSETS				
Debtors and prepayments	2,241		41,958	
Cash at bank and in hand	413,382		387,268	
	-----		-----	
Total current assets	415,623		429,226	
	-----		-----	
CREDITORS				
Amounts falling due within one year	2,220		5,930	
	-----		-----	
Net current assets		413,403		423,296
		-----		-----
NET ASSETS		413,403		423,296
		-----		-----
FUNDS OF THE CHARITY				
Restricted funds		-		-
Unrestricted general funds		14,522		18,109
Unrestricted designated funds		398,881		405,187
		-----		-----
		413,403		423,296
		-----		-----

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 ..16/9/2025..... 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 2024

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 2024

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

	2024 £	2023 £
Research	89,453	16,151
Independent examiner's remuneration	1,000	1,000
	-----	-----

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

	2024 £	2023 £
Sick Kids	36,036	-
Hnia at Inserm	-	9,958
Inserm(Buj Bello) 2023-25	32,682	4,167
Great Ormond Street (GOSH)	20,735	-
Newcastle University re Treat NMD	-	2,026
	-----	-----

5. DEBTORS AND PREPAYMENTS

	2024 £	2023 £
Gift Aid	1,241	458
Prepayments and accrued income	1,000	41,500
	-----	-----
	2,241	41,958
	-----	-----

6. CREDITORS FALLING DUE WITHIN ONE YEAR

	2024 £	2023 £
Accruals	2,220	5,930
	-----	-----

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 DECEMBER 2024

7. RELATED PARTY TRANSACTIONS

There were no related party transactions by the trustees for the year (2023: None).

One Trustee received remuneration of £20,000 during the year ending 31 December 2024 (2023: £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.