



Annual Report and Financial Statements

To the year ended
31 December 2022

myotubular trust
FINDING STRENGTH



UK Registered Charity, 1137177
A Company Limited By Guarantee

TRUSTEES' ANNUAL REPORT 31 December 2022

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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 2022.

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 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. 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 three 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 in particular, 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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2022 – where are we now

In the 17 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. Substantial progress has been made in understanding myotubular and centronuclear myopathy, and research into a treatment, or cure, has made great steps forward, including the first human clinical trials.

However, there remain many questions about the condition, and why and how these genetic mutations, and the proteins they impact, cause muscle weakness. There have been deeply disappointing setbacks in the clinical trials – the gene therapy trial is still on hold after the death of four participating patients; and the ASO trial (explained further below) was terminated due to concerning liver test results. Combined with the history of some (but not all) of those with XLMTM 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 in order 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. 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 **£2.6M**, through family-led fundraising, networks and relationships.
2. Made **17 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 (in person) family conferences**, bringing together affected individuals and families, researchers, clinicians, and other health professionals, and arranged several family meetings by **Zoom during the pandemic and beyond** 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. Created a great band of **committed supporters**, raising funds for us year on year.
14. 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.
15. And **most importantly**, brought together **families** affected by myotubular and centronuclear myopathy, who support the Trust's fundraising and each other's lives.

In 2022 specifically...we have:

1. Raised nearly £150,000.
2. Invited applications for our 13th research grant round, this time in partnership with Muscular Dystrophy UK.
3. Awarded a grant to Dr Ana Buj Bello at Genethon, Paris, to develop an even more specific and potentially safer gene therapy.
4. Promoted, and participated in, a focus group of UK patients and parents as part of *Lessons Learnt from Clinical Studies in Centronuclear Myopathies*, an international study led by Radboud University, the Netherlands, and initiated at the 2022 family conference in Germany, organised by the German patient organisation ZNM-ZusammenStark.
5. Presented at the **MHRA One Agency** annual 'away day', as one of the two charities presenting the patient perspective to the whole staff of the UK drug regulator.
6. Launched, in collaboration with University College Cork, a Home Care survey to gather information on home care packages available in the UK, to support newly diagnosed families with real word evidence.
7. Supported the launch of a 'Burden of Care' survey to the UK myotubular and centronuclear myopathy caregiver community, as part of Astellas Pharmaceutical's gathering of data that will ultimately be used as part of any submission (from any company) to NICE for drug or treatment approval.

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8. Collaborated 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.
9. Facilitated the development of a Liver Questionnaire, the first new section of the MTM & CNM Patient Registry, again with MTM-CNM Family Connection and the expertise of the Liver Collaborative Working Group.
10. For the 16th year since founding, we have raised 100% of our running costs separately from the funds raised by families and supporters, meaning that once again we meet our ambition to dedicate all the funds raised by supporters into research.

Family Support in 2022

Advocacy and Family Support

In the 17 years from launch in 2006 to 2022 we are regularly reminded that specific support is needed for myotubular and centronuclear myopathy patients and families. This is, in the main, because centronuclear and myotubular myopathy are so rare that 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.

In 2022 we launched a survey, a collaboration between the Myotubular Trust and University College Cork, to gather reliable, objective, analysable data on home care packages in place for myotubular and centronuclear myopathy patients throughout the UK. With that information we hope to be able to support new families and their healthcare teams at a very difficult time.

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 more often 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. Shared experiences and information signposting are needed again and again by families. In 2022 we 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.

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Research in 2022

The Myotubular Trust was set up to raise funds to leverage the 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. It began recruiting 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. The trial 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.

The mTORC pathway

Dr Karim Hnia at the world-renowned French institute, INSERM, was awarded a 2-year grant in 2020 by Myotubular Trust investigating the question "Is the mTORC1 pathway a route to treatment in x-linked myotubular myopathy?"

mTORC1 is a master regulator of muscle growth – from the early steps of muscle cell differentiation in the womb, to the work of muscles in adult life. The work of Dr Hnia's laboratory has shown that mTORC1 is 'overactivated' when XLMTM is present. What makes this a very interesting project (apart from the highly positive peer review) is that there are a number of drugs already licensed to regulate mTORC1 levels for other diseases.

This grant finished in 2022, having shone new light on how myotubularin controls muscle growth signalling during cell development. This opens up the possibility of identifying drugs that can trigger specific muscle signalling. We anticipate that this work will be published in 2023/24.

Next generation gene therapy

Dr Ana Buj Bello was the recipient of the first ever research grant from the Myotubular Trust for her pioneering work on gene replacement therapy for xlmtm. The goal of this 2022 grant to Dr Buj Bello and her team at Genethon is to develop an even more specific and potentially safer MTM1 vector to be brought to clinical trial. They 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 will generate optimised vectors with increased potency and specificity for MTM1 gene delivery.

Patient Registry

In order to add the new liver section to the MTM and CNM Patient Registry the Myotubular Trust, together with the MTM-CNM Family Connection in the US, provided a grant to the Newcastle University Registry team to fund the software development and project management of these new questions. It was heartening to see how quickly we could make this happen as a team united behind a very important purpose. The new section will be ready to launch early in 2023.

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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. *"It is the result of perseverance and joined forces. Thanks a lot for your support all along these years, your contribution has been crucial, you can be proud of it"* Dr Buj Bello

Dosing patients in this trial began in 2018, and 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 it is now 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.

Antisense Therapy

The 3-year grant, *"Reducing DNM2 as a novel therapeutic target for CNM"*, made by the Myotubular Trust to Jocelyn Laporte's team at the laboratory IGBMC in 2016 *"validated a deliverable approach"*, and formed a major contribution to the pre-clinical studies preparing the way for a clinical trial to test this approach in humans who have either the x-linked MTM or Dynamin 2 form of the condition.

In 2019, Dynacure, a start-up biotech based in Strasbourg, secured approval for a clinical trial for those affected by the MTM1 and DNM2 genes, and this trial was due to begin in 2020. One patient began treatment, but unfortunately the pandemic put the trial on pause for a number of months. However, by the end of 2020 recruitment was restored, and initial dosing began in several countries in Europe, including in the UK. This progressed throughout 2021, but to all our shared disappointment, the trial was halted in the autumn of 2022 due to the reporting of adverse events, specifically elevated liver test results and low blood platelet counts. These were ultimately resolved for each patient by stopping treatment, but the company decided that they could no longer pursue this work without understanding why the liver seems to be affected in this condition.

'This is not the news we ever wanted to share, as we know how great your needs are for safe and effective treatments. As a company, and as individuals, we are overwhelmed with disappointment' Chris Freitag, Chief Medical Officer, Dynacure.

One of our major learnings through this heart-breaking process was the value of our rare disease data. It is not unusual when a clinical trial is halted, for all the data to be abandoned. However, the researchers involved both from the company, and the Principal Investigators from specific trial sites, were committed to saving, using and publishing as much data as possible. In the months after the trial stoppage was announced, and before the company formally terminated the project, they worked together to develop ways to save and store the data, and routes to publication. We know now that this is not usual, and we are very grateful to those individuals involved.

We also sincerely thank those affected by myotubular and centronuclear myopathy who took part in this trial. 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

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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, for adding to the sum knowledge of our understanding of the condition.

Tamoxifen clinical trial

Setting up this trial has had a number of setbacks, with Covid having a not insubstantial 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, 2022 was a year where as team – researchers, clinicians and patients - we worked hard to push to solve a number of blockages, and as the year ended everything was ready for the trial to begin recruitment in the UK early in 2023.

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 these 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. 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, 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.

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Financial Review

Sustainable Fundraising – the Highlights of 2022

There is no doubt that the post Covid fundraising landscape has changed fundamentally, and therefore the Trustees were very grateful that we were able to raise in the region of £150,000 once again this year - £146,024 to be precise. Although challenge-type amateur sporting events have come back to some degree since Covid, willingness to look for sponsorship seems to have reduced, based on our experience over the last 16 years. 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.

2022 was the year of:

- The 16th Jack Blunsdon Golf Day
- A 100km walk from Team Robin
- A New Year sea swim in the cold waters off Ireland!
- The Six Peaks Challenge team
- Summer festival in Germany and evening event in Northern Ireland
- A brave London Marathon runner
- The Manchester intrepid Half Marathon runner
- Birthday donations
- Everest Trek by Team Noah
- A School dress down day
- Christmas donations in lieu of cards

We also were delighted to receive an Amazon Community UK donation following a nomination by a member of Team Robin.

Sadly, 2022 was another year of many losses and we extend our gratitude to the families and friends of those boys who, in their time of grief, considered the Myotubular Trust for donations.

We were fortunate that the generous post-Covid funding from the Global's Make Some Noise appeal continued through 2022 and we cannot thank them enough for their interest in our small charity, and the care they took of us. Speaking of financial and moral support, a special mention goes once again to IGY Foundation who are so good to us, when we fully realise there are many causes who must look to them for help. Thank you for seeing the value of the work we do.

Once again we had an increase in standing orders during 2022, probably as people realised how difficult it was for charities during social distancing and lockdowns. Thank you to you all.

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.

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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.

Plans for future periods

The Trust aims in 2023 to:

- Maintain excellence in family support, advocacy, information provision, and continue to make the introductions that families tell us are invaluable.
- Finalise the development, and actively promote and support the launch, of the Liver Questionnaire on the Myotubular and Centronuclear Myopathy Patient Registry.
- Organise 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.
- Facillate and support the development of liver related resources for patients to share with their medical teams, and liver data to share with the clinical and research communities.
- Award a grant (or grants) to further the 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.
- Share the publication of the work of Dr Karim Hnia on mTORC signalling in mtm.
- 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.

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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

Greg Fowler (resigned 22 September 2022)

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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Bankers

CAF Bank Limited

Kings Hill

West Mailing

Kent

ME19 4JG

The Co-operative Bank

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Skelmersdale

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Independent Examiner

Michael Stone MA ACA

Jamieson Stone LLP

Windsor House

40/41 Great Castle Street

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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 2022

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 2015) 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



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25/9/2022

STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2022

	Unrestricted Funds £	Designated Funds £	Restricted Funds £	Total Funds 2022 £	Total Funds 2021 £
<u>INCOMING RESOURCES</u>					
Donations and grants	43,159	64,340	-	107,499	101,003
Fundraising activities	38,525	-	-	38,525	43,028
Interest received	1,438	-	-	1,438	739
Total incoming resources	83,122	64,340	-	147,462	144,770
<u>RESOURCES EXPENDED</u>					
Research	-	40,704	-	40,704	41,289
Fundraising costs	1,285	-	-	1,285	464
Outreach work	-	-	-	-	2,500
Staff costs	49,961	-	-	49,961	49,961
Training	-	-	-	-	719
Advocacy	-	-	-	-	13,000
Family Conference costs	-	-	-	-	150
Accountancy & payroll	300	-	-	300	140
Independent examiner	1,000	-	-	1,000	1,000
Legal & professional	943	-	-	943	445
Trustee's indemnity	917	-	-	917	873
Travel costs	183	-	-	183	-
Printing & stationery	107	-	-	107	89
Postage & telephone	79	-	-	79	11
Subscriptions	252	-	-	252	174
Web & IT costs	1,658	-	-	1,658	1,497
Bank charges	236	-	-	236	214
Miscellaneous costs	319	-	-	319	104
Total resources expended	57,240	40,704	-	97,944	112,630
Net incoming/(outgoing) resources	25,882	23,636	-	49,518	32,140
Transfers between funds	(26,260)	26,260	-	-	-
Net movement in funds	(378)	49,896	-	49,518	32,140
Total funds brought forward	6,701	320,442	-	327,143	295,003
Total funds carried forward	6,323	370,338	-	376,661	327,143

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 2022

	£	2022 £	£	2021 £
CURRENT ASSETS				
Debtors and prepayments	32,030		25,419	
Cash at bank and in hand	348,676		303,447	
Total current assets	380,706		328,866	
CREDITORS				
Amounts falling due within one year	4,045		1,723	
Net current assets		376,661		327,143
NET ASSETS		376,661		327,143
FUNDS OF THE CHARITY				
Restricted funds		-		-
Unrestricted general funds		6,323		6,701
Unrestricted designated funds		370,338		320,442
		376,661		327,143

The company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 December 2022 and the members have not required the company to obtain an audit of its financial statements for the year ended 31 December 2022 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 25/9/2023 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 2022

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 2015) 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 2022

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

	2022 £	2021 £
Research	40,704	41,289
Independent examiner's remuneration	1,000	1,000
	<u>41,704</u>	<u>42,289</u>

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

	2022 £	2021 £
Hnia at Inserm	38,929	29,059
Tamoxifen Protocol	-	2,469
Newcastle University re Treat NMD	-	7,500
Research Consultancy	1,775	2,261
	<u>40,704</u>	<u>39,289</u>

5. DEBTORS AND PREPAYMENTS

	2022 £	2021 £
Gift Aid	1,530	419
Prepayments and accrued income	30,500	25,000
	<u>32,030</u>	<u>25,419</u>

6. CREDITORS FALLING DUE WITHIN ONE YEAR

	2022 £	2021 £
Accruals	4,045	1,723
	<u>4,045</u>	<u>1,723</u>

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 DECEMBER 2022

7. RELATED PARTY TRANSACTIONS

There were no related party transactions by the trustees for the year (2021: None).

One Trustee received remuneration of £20,000 during the year ending 31 December 2022 (2021: £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.