



## **Annual Report and Financial Statements**

To the year ended  
31 December 2020



# TRUSTEES' ANNUAL REPORT 31 December 2020

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## TRUSTEES' ANNUAL REPORT 31 December 2020

The Trustees of the Myotubular Trust take pleasure in presenting their annual report and the financial statements for the year ended 31 December 2020.

### 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, its Trustees, staff and many volunteers all focus 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 advocacy, 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 – called this because 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, boys and girls and are generally milder in severity, although not always. The terms myotubular and centronuclear myopathy both describe 'our' condition.

Children and adults with myotubular myopathy often require vigilant nursing care, ventilators to breathe, power wheelchairs to get around, and are usually 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.

In 2020, three young boys who were taking part in the gene therapy trial for x-linked myotubular myopathy tragically died. We would like to take this opportunity to extend the deepest sympathies of the Myotubular Trust to their families and those who loved and took care of them.

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## Impact of COVID-19: Uncertain times

The impact of the global pandemic on the myotubular and centronuclear myopathy patient community was undoubted. This group of patients was identified as '**at risk of severe illness if they catch Coronavirus**' and were generally advised to shield. The effect of this new virus on those with a neuromuscular condition was of course unknown at the outset and it was a hugely concerning time. Happily, the severity of the disease did not seem to be worsened for our group of patient and we did not experience an increased death rate due to Covid19.

The initial NHS response in the UK to our patients and families regarding advice was very thorough. Families and individuals felt that they were provided with good signposting and helpful information around shielding, accessing emergency food and medicines.

However, there were a myriad of other ways in which the myotubular and centronuclear myopathy patient and family community were affected by the pandemic in 2020 including;

- Delays with planned routine treatments and health screening.
- Postponement of surgical interventions, which while not immediately urgent, were vital.
- Complexities, and profound delays, in discharge from hospital for new babies.
- An increased care load for parent caregivers who either chose, for safety reasons to cease having carers and nurses come into the home, or whose carers were unable to work (due to homeschooling their own children; self-isolation requirements; lack of PPE; risk of cross contamination between their caseloads).
- Getting sufficient respiratory related medical supplies and getting hold of PPE in the first months.
- A shortage of skilled agency staff with in-depth respiratory and ventilation training.
- Lack of clear guidelines about ventilated children returning to school – often meaning they could not go back with their classmates.
- Ongoing anxiety about mixing socially, and therefore increased isolation for an already isolated group.
- The pausing of clinical trials and studies – for a disease which has waited a very long time for any human clinical trials.

*"We lost a few nurses due to COVID related complications in their own lives. So now I step in to fill the gaps, and lack of sleep has been a big issue in terms of holding it together."*

*"My son was happy staying home during lockdown, and we have struggled getting him out and about since - he wants to stay home and do home schooling and play with his Wii. His big sister struggled going back to work and had big anxieties about bringing Covid home to her brother. I had to quit my job because of fear of getting Covid and bringing it home."*

*"My husband and I got Coronavirus at the same time, and I was so scared that my son's carers would walk away. But actually, they were amazing and increased their working hours."*

The Myotubular Trust responded to the pandemic related needs of our community by:

- Offering all the support we would normally have provided in person by phone and conference call facility, including sometimes training families in the use of online tools.
- Ensuring that the most up to date information for those living with NMD was available on the website as soon as it became available.
- Organising Zoom Family meetings - covering topics including research updates, studies on carrier women and mental health and well-being, delivered by a range of professional experts and neuromuscular scientists and clinicians.
- Finding online fundraising events for those who wanted to fundraise or wanted to have a goal for activity.

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## Achievements and Performance

Myotubular Trust is now 15 years old, and we are gratified to find that we continue to make a real and meaningful difference, even in remarkably unprecedented times. There is clearly a very useful role for a disease specific research funder in the world of rare disease, along with a tangible need to support patients and families

The Trust was established in 2006 by two parents of boys with myotubular myopathy, to raise research funds. We had found that 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.

When Myotubular Trust was established, we hoped to be able to fund one or two substantial pieces of research. We hoped that we would help establish proof of principle in one or two key areas, perhaps fund research to start to answer certain important unknowns and 'compete for brains' in a field where it was clear that breakthroughs were on the horizon.

Little did we realise that the Trust would still be a viable fundraising and funding resource 15 years later; how we would be the charity that funded the proof of principle research that led to the first ever clinical trial for x-linked myotubular myopathy; how many different and fundamental research possibilities would open up; how many families would come together to provide support for each other; how much we would see myotubular and centronuclear myopathy push ahead of other rare neuromuscular diseases in bringing research to pre-clinical studies and clinical trial; and finally ...how we would see the charity and community through a global respiratory pandemic.

### Since founding 15 years ago...we have:

1. Raised **£2.4M**, through family-led fundraising, networks and relationships.
2. Made **16 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 which began in the autumn of 2017.
4. Funded **other proof of principle projects** that are on track to lead to clinical trials.
5. Funded two 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 more family meetings by **Zoom during the pandemic 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. Sponsored a **Standards of Care** meeting for clinicians, patients and their healthcare professionals at the Royal Brompton Hospital.
9. Participated in a European Neuromuscular Centre workshop on Mouthpiece Ventilation, resulting in the publication of **MPV international best practice guidelines**.
10. Created a great band of **committed supporters**, raising funds for us year on year.
11. Developed strategic relationships with **key stakeholders** in the fields of neuromuscular disease, rare disease, EU and UK regulatory bodies, and highly regarded well-established UK children's charities.
12. And **most importantly**, brought together more and more families affected by myotubular and centronuclear myopathy, supporting the Trust's fundraising and each other's lives.



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## *Family Support in 2020*

### **Advocacy and Family Support**

We were very fortunate that since 2016-2017 we have received annual grants from IGY Foundation to enable us to support families and to help with professional advice on respiratory issues, ventilatory support, applications for home services and adaptations, school support etc. This service was invaluable in the challenging year 2020.

As a reminder of why this support is needed, myotubular myopathy is so rare that usually the child'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 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. For example:

- 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 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 to take 1 year to 18 months. There are huge differences between the capability of different hospitals, different clinical commissioning groups, and different community nursing support services in making this happen in a timely fashion. This was further complicated in 2020 by the challenges of bringing together the multi-disciplinary teams necessary for this complex step, and the high demand for respiratory and ventilatory trained agency staff.
- Parents also have a desperate need for encouragement, reassurance, and practical support to feel comfortable with their new parent caregiving roles quickly, to help them better understand the nuances of the condition and to help them to feel more 'in control'.
- Local medical teams, who are quite likely seeing their first ever baby with myotubular myopathy, need support with information and access to specialist advice and support - such as what equipment to provide the family and what the optimal settings may be on breathing equipment.

The need 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 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. So often experiences and information signposting are needed again and again by families.

We were very glad to be able to provide this support to many families again in 2020 despite the challenges of a global pandemic.

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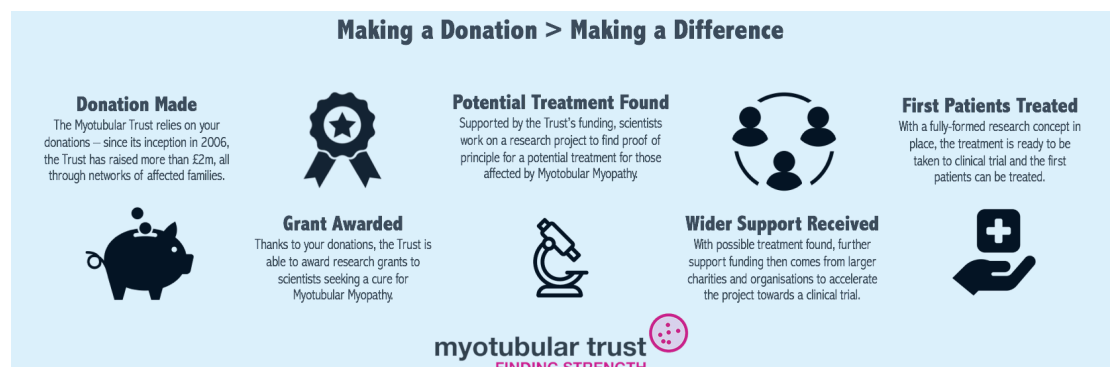
### Case study

The advice given to the parents of a 2 year old boy with myotubular myopathy has had a huge influence on how they manage his airways to keep him safe. In the early days, when the baby was unable to cope with the volume of secretions and he wasn't strong enough to swallow, we drew his parent's attention to nasal suctioning, chest physiotherapy and nebulisers. This was at a point when their hospital was not putting these steps in place as they were unfamiliar with the condition. Myotubular Trust also advocated for the family to use a cough assist device, which is now an integral part of the boy's physiotherapy routine - this has strengthened his cough and improved his ability to clear his secretions independently. This is all second nature to his parents now, and together with the night-time nursing care package that the Trust helped to put in place, his parents believe that the airways management regime is the reason their son has avoided chest infections and lengthy hospital admissions in the first two years of his life. They say that it is also why, at the age of 3, he is strong enough to attend nursery, just like any other little boy his age.

*"Myotubular Trust has been there, and will continue to be there, at every stage of this journey we are on, to support us and improve his life experience and that is incredibly comforting and reassuring to us as parents. I really couldn't ever state this enough!!"*

### Research in 2020

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. This we do by investing in the very best translational research, peer reviewed to the highest international standards, supported by an eminent Scientific Advisory Board.



### Proof of principle funding

The founding vision for the Trust was to fund proof of principle projects that would not normally be funded by public or industrial funding. For rare diseases it can be incredibly difficult to finance this vital stage of research, but once proof of principle is established for a potential treatment, then other larger charities and organisations are more likely to provide funds for the next stage. We had great hopes that this strategy might make some difference for our very rare disease, and we are gratified that all three current promising clinical trials for treatments have had significant early-stage funding from Myotubular Trust.

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### Clinical trials

#### *Gene therapy*

This virus mediated gene therapy was being funded by the 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 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

This trial reached its 3 year mark in 2020, but tragically and devastatingly 3 young boys died, probably due to liver complications. The trial was put on hold by the FDA in the US for most of 2020, and after some months of reporting by the sponsoring company (previously Audentes Therapeutics, now part of Astellas Gene Therapies), the hold was lifted again in December. In 2021 it is expected that dosing will resume, at a lower dose than that given to the deceased boys.

#### *Antisense Therapy*

The 3year 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.

#### *Drug repurposing*

2020 was the third year of our 3-year drug repurposing research grant to Dr James Dowling at the Hospital for Sick Children, Toronto, Canada. One of the aims of this grant was to determine the optimum dosage of Tamoxifen to take to clinical trial. By the end of 2020 this work had progressed successfully enough for clinical trial planning to be well advanced in sites in Canada, the US, and the UK (see grant announcement below).

Drug repurposing has the benefit of bringing drugs that have already been safely taken by thousands, maybe millions of other people to a new group of patients. As safety has already been established, the length of clinical trial required to test such a drug for a new patient group, and the proof required for the regulatory authorities, can be much reduced. Patients, particularly those already living with a high degree of vulnerability, may also feel a lot more confidence and faith in a known drug.

This now brings **the number of clinical trials** for centronuclear and myotubular myopathy to **three**, all of which have had substantial investment and commitment from Myotubular Trust. On the road to all of 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 Audentes (now 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.

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### Grant awards – 2020

In 2020, despite the challenging fundraising and financial position of charities throughout the UK, we were in the fortunate position to be able to make two research 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 well the well-known oncology drug, Tamoxifen, works in improving motor and respiratory function in patients with myotubular myopathy. The trial will be led by Dr Giovanni Baranello, and Professor Francesco Muntoni at the UCL Great Ormond Street Institute of Child Health. It will involve children affected by x-linked myotubular myopathy in the UK and is part of the global repurposing trial from the work the Trust funded with Dr Jim Dowling at Sick Kids Canada. The global trial is being led by Dr Dowling.

#### *The mTORC pathway*

Our second grant award of 2020 went to **Dr Karim Hnia** at the world-renowned French institute, INSERM, 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 through other diseases there are a number of drugs already licensed to regulate mTORC1 levels.

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Another important goal in being a source of funding for this condition was to inspire others in the field to work on myotubular and centronuclear myopathy in order to develop a sustainable legacy of research for our condition. Fourteen years later we are delighted to be supporting a committed community of researchers with an interest in myotubular myopathy, undertaking world-class research projects.

We are also immensely grateful to our Scientific Advisory Board (SAB) for their time and their highly impressive 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 Duchon, 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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### *Partnerships*

With the goal of collaboration and innovation in mind, we continued in 2020 to work with key stakeholders in the research, children's charity, industry, regulatory and myotubular myopathy communities:

- The research charity of the world-renowned children's hospital, Great Ormond Street, again invited us to partner with them to issue a joint grant call for translational research into myotubular and centronuclear myopathy, and together we are jointly funding a clinical trial to examine the effect of Tamoxifen on the symptoms of x-linked myotubular myopathy.
- We took part in the creation and promotion of a European study, led by the University of Essen, into symptoms of muscle disease in female carriers and relatives of boys with x-linked myotubular myopathy. This study was initiated at the Family Meeting of the German patient organization ZNM – Zusammen Stark e.V.
- We supported the communications of the centronuclear myopathy voluntary organisation, The Information Point.
- We supported lobbying initiatives by MDUK, Genetic Alliance UK and Rare Disease UK in parliamentary meetings.
- We contributed to meetings of the Patient Group Consultative Forum of the UK Medicine and Healthcare Products Regulatory Agency (MHRA).
- We continued to engage in learning and dialogue about Highly Specialised Technologies with NICE, the UK public body which provides advice and guidance on new medicines to be adopted by the NHS.
- We continued to work closely with organisations in the biotech field who are focusing on myotubular myopathy; the TREAT-NMD's Registry team; and with corporate backers who provide us with pro bono services or grants for running the Trust.

Given our relatively small size, these partners are crucial for us and we value their time and commitment immeasurably, and hope we can continue to be excellent partners with them.

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## Financial Review

### *Sustainable Fundraising – the Highlights of 2020*

In 2020 the Trustees were gratified that we were able to raise £131,865, despite the remarkably unprecedented times impacting charity fundraising. Our continued revenue stream is testament to the strength of support that a small number of affected families enjoy in their communities – the 'ripple effect' from each child into an extended network of supporters has been so critical to our growth and stability.

Despite the year that was in it, our families were able to organise

- The 14<sup>th</sup> Jack Blunsdon annual golf tournament – with full social distancing
- Two 10K runs
- A half marathon
- Several events to support the 2.6 Challenge – a virtual fundraising call to action, arranged by the organisers of the London Marathon, suggesting that people raise money for charities by creating any event that involved the numbers 2 and 6 (in recognition of the length of a normal marathon)
- In memoriam donations
- Online shopping and eBay donations
- As well as a range of 10K runs, Hope Walks, Tough Mudders, a very impressive London Marathon run and a first ever Manchester Marathon.

We also had an increase in standing orders during 2020, probably as people realised how difficult it was for charities during social distancing and lockdowns. Finally, we were incredibly fortunate that JCW Resourcing, an IT recruitment company, chose the Myotubular Trust as the recipient of their annual January fundraising event, in recognition of the lovely son of one of their colleagues. This provided us with a very welcome £30K+ start to our fundraising in 2020 - little did we know then how vital that would be.

We were also lucky to be chosen by the charity, Global's Make Some Noise for emergency Covid funding. Their process was very thoughtful and considerate, and we really felt that there were organisations out there rooting for small charities like ours.

We would also like to extend our grateful thanks to the IGY Foundation for their continued support of our family support work – we are truly grateful to them for both the funds, and for the interest in, and understanding of this work we do.

### *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).

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### **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 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 and prepare to announce another grant call for 2021.

### **Plans for future periods**

The Trust aims in 2021 to:

- Maintain excellence in family support, advocacy, information provision, and continue to make the introductions that families tell us are invaluable.
- Announce our 12<sup>th</sup> call for grants.
- Continue to work with key stakeholders in the neuromuscular disease community, and strategic rare disease organisations.
- Continue to work with the regulatory authorities on behalf of patients with myotubular and centronuclear myopathy – both the MHRA in the UK and the EMA in Europe.
- Plan ahead and prepare effectively for any future NICE review of treatments for myotubular and centronuclear myopathy, facilitating families' and patients' input.
- Partner with, and support TREAT-NMD on the long-term excellence and sustainability of the Myotubular and Centronuclear Myopathy Patient Registry.
- 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.
- Maintain our 2020 support of fundraising events, despite the continued challenges brought about by the pandemic.

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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  
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

## 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 2020

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## 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

*Michael Stone*

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

20 August 2021

## STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2020

	Unrestricted Funds £	Designated Funds £	Restricted Funds £	Total Funds 2020 £	Total Funds 2019 £
<b><u>INCOMING RESOURCES</u></b>					
Donations and grants	85,628	25,000	-	110,628	170,147
Fundraising activities	20,635	-	-	20,635	47,994
Interest received	1,602	-	-	1,602	1,468
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Total incoming resources	107,865	25,000	-	132,865	219,609
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<b><u>RESOURCES EXPENDED</u></b>					
Research	-	133,306	-	133,306	79,500
Fundraising costs	345	-	-	345	2,953
Staff costs	50,765	-	-	50,765	51,020
Training	-	-	-	-	81
Advocacy	-	12,187	-	12,187	13,891
Family Conference costs	-	-	-	-	-
Accountancy & payroll	110	-	-	110	280
Independent examiner	1,000	-	-	1,000	1,000
Legal & professional	-	-	-	-	-
Trustee's indemnity	966	-	-	966	865
Travel costs	216	-	-	216	1,403
Printing & stationery	72	-	-	72	337
Postage & telephone	56	-	-	56	140
Subscriptions	260	-	-	260	160
Web & IT costs	688	-	-	688	625
Bank charges	300	-	-	300	497
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Total resources expended	54,778	145,493	-	200,271	152,752
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Net incoming/(outgoing) resources	53,087	(120,493)	-	(67,406)	66,857
Transfers between funds	(51,194)	51,194	-	-	-
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Net movement in funds	1,893	(69,299)	-	(67,406)	66,857
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Total funds brought forward	4,069	358,340	-	362,409	295,552
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Total funds carried forward	5,962	289,041	-	295,003	362,409
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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

Myotubular Trust

## BALANCE SHEET AS AT 31 DECEMBER 2020

	£	2020 £	£	2019 £
CURRENT ASSETS				
Debtors and prepayments	26,275		1,561	
Cash at bank and in hand	270,455		364,280	
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Total current assets	296,730		365,841	
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CREDITORS				
Amounts falling due within one year	1,727		3,432	
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Net current assets		295,003		362,409
		-----		-----
NET ASSETS		295,003		362,409
		-----		-----
FUNDS OF THE CHARITY				
Restricted funds		-		-
Unrestricted general funds		5,962		4,069
Unrestricted designated funds		289,041		358,340
		-----		-----
		295,003		362,409
		-----		-----

The company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 December 2020 and the members have not required the company to obtain an audit of its financial statements for the year ended 31 December 2020 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 ..20th August.2021..... and signed on their behalf:

*Andrew Lennox*

.....  
Andrew Lennox  
Chairman/Trustee

*James Rosling*

.....  
James Rosling  
Treasurer/Trustee

The accompanying notes form part of these financial statements

# NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2020

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## 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 2020

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#### 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

	2020 £	2019 £
Research	133,306	79,500
Independent examiner's remuneration	1,000	1,000
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The principal research grants awarded during the year were as follows:

	2020 £	2019 £
Sick Kids Canada - Dr Dowling	72,000	72,000
Sparks Charity GOSH	58,108	-
Taurus Tide	3,198	-
Newcastle University re Treat NMD	-	7,500
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#### 5. DEBTORS AND PREPAYMENTS

	2020 £	2019 £
Gift Aid	133	175
Prepayments and accrued income	26,142	1,386
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	26,275	1,561
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#### 6. CREDITORS FALLING DUE WITHIN ONE YEAR

	2020 £	2019 £
Accruals	1,727	3,432
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## **NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 DECEMBER 2020**

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### **7. RELATED PARTY TRANSACTIONS**

There were no related party transactions by the trustees for the year (2019: None).

One Trustee received remuneration of £20,000 during the year ending 31 December 2020 (2019: £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.