



Duchenne Research Fund

THE DUCHENNE RESEARCH FUND

**REPORT AND FINANCIAL STATEMENTS
FOR THE YEAR ENDED 31 DECEMBER 2019**

Registered Charity No. 1119068

**AZETS AUDIT SERVICES
Greytown House
221/227 High Street
Orpington
Kent BR6 0NZ**

The Duchenne Research Fund partners with brilliant scientists and innovative biotech companies to accelerate treatments for Duchenne muscular dystrophy. Help us find a cure for this devastating disease.



“Cycling for kids who can’t”

#TeamDRF cyclists raised funds for Duchenne research at the Prudential RideLondon-Surrey cycle ride on 4 August 2019

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REFERENCE AND ADMINISTRATIVE DETAILS

Trustees	Daniel Baum Simon Kanter Jeremy Shebson Sarah Shelley
Charity Registration Number	1119068
Principal Office	Symal House 423 Edgware Road London NW9 0HU
Key Management	Trustees
Banker	Bank of Scotland Teviot House 41 South Gyle Crescent Edinburgh EH12 9DR
Investment Managers	Canaccord Genuity Wealth Management 41 Lothbury London EC2R 7AE
Statutory Auditor	Azets Audit Services Statutory Auditor Greytown House 221 – 227 High Street Orpington Kent BR6 0NZ

REPORT OF THE TRUSTEES

The Trustees present their report and audited financial statements for the year ended 31 December 2019.

The financial statements have been prepared in accordance with the accounting policies set out in note 1 to the accounts and comply with the charity's trust deed, the Charities Act 2011 and Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK published on 16 July 2014.

Objectives

The charity was founded to:

'Promote the relief of persons suffering from and to promote research into the treatment, cure, care and welfare of those persons suffering from Duchenne muscular dystrophy in England, Wales and Overseas.'

ABOUT THE DUCHENNE RESEARCH FUND

The Duchenne Research Fund (DRF) was founded with a clear and ambitious goal: that, through our work and the research we are funding, a cure will be found for Duchenne muscular dystrophy.

Duchenne muscular dystrophy is a severe and progressive fatal muscle wasting disease that almost exclusively affects boys. Sufferers lose the ability to walk, and are confined to a wheelchair by their early teens. By their early 20s these boys are left fighting for their lives as the muscles of their lungs and heart deteriorate. This devastating condition is fatal for all sufferers, and there is currently no cure. It claims the lives of 100 young people every year in the UK alone.

The DRF identifies and funds research into potential treatments and cures, with the aim of improving the condition of all boys living with Duchenne – now and in the future. We focus on creating powerful collaborations between some of the world's most brilliant scientists, proactive Duchenne charities, and innovative biotech companies to accelerate the development of therapies that are ripe for treating Duchenne. We aim to specifically focus our efforts on research related to the pathogenesis and treatment of Duchenne muscular dystrophy for all boys regardless of mutation.

For more information about Duchenne muscular dystrophy and the Duchenne Research Fund, please visit our website at www.duchenne.org.uk

FUNDRAISING IN 2019

PRUDENTIAL RideLondon-Surrey

Following the success of the Prudential RideLondon-Surrey event in 2018, DRF retained places in the 2019 event. Despite the event being moved into August in 2019, at a time when many supporters were away or unable to take part, we are grateful to those who took up the challenge and together raised £5,000.

Due to the Covid-19 pandemic, the 2020 event has been cancelled. The organisers have confirmed that the 10 event places already paid for will be converted to five London Marathon places for 2021. The 2021 London Marathon is scheduled for October 2021.

OTHER FUNDRAISING ACTIVITY

The DRF is reliant on fundraising to support its investment in research projects and to raise awareness of this life-threatening disease, and all donations are gratefully received. There have been several fundraising initiatives throughout the year undertaken by many of our supporters, including birthday gifts and significant general donations from existing and new supporters. We are extremely grateful for the efforts of many who have given so much of their time and organised various initiatives. Due to the Covid-19 pandemic, the DRF has no in-person events planned for 2020. This will be reviewed as the situation evolves.

Please contact info@duchenne.org.uk with any suggestions or ideas for events or fundraising activities that you think will help the charity.

REPORT OF THE TRUSTEES (CONTINUED)

OTHER INCOMING FUNDS

In 2017 Solid Biosciences reported that it had raised up to \$50 million to continue to advance its programs for Duchenne, following which the trustees decided (jointly with two other UK Charities with which the DRF co-invested) to realise some of DRF's investment which has enabled DRF to pursue other avenues of research and programmes as advised by our Scientific Advisory Board. The balance of the funds from this sale of shares were transferred to the charities (including the DRF) in 2019. In 2018 Solid Biosciences completed an IPO on the NASDAQ stock exchange. The remainder of the charities' holdings has been retained in Solid Biosciences.

The DRF is committed to continuing to raise funds to tackle every aspect of this multi-faceted disease. We will continue to look for ways in which we can support Duchenne therapies moving to clinical trials as quickly and safely as possible, in the search for a cure. We will seek out complementary therapies that aim to ensure as many boys and young men as possible will be eligible to receive curative treatments if and when they become available. We also want to ensure that all boys, whether ambulant or non-ambulant, are eligible to participate in clinical trials and get access to vital care and treatments.

To stay up to date with our latest news, visit www.duchenne.org.uk/news, or sign up for our newsletter via the homepage.

FUNDING STRATEGY IN 2019

The DRF has its own Scientific Advisory Board, comprising some of the leading minds in the field of muscular dystrophy. This board is spearheaded by Dr Ronald Cohn (Paediatrics, University of Toronto; President and CEO of the Hospital for Sick Children, Toronto, where he is also Chief of Clinical and Metabolic Genetics and Co-director of the Centre for Genetic Medicine. He is joined on the board by Dr Kevin Campbell (Professor of Physiology and Biophysics at the University of Iowa), Dr James Dowling (Senior Scientist at the Hospital for Sick Children, Toronto, and Associate Professor of Paediatrics and Molecular Genetics at University of Toronto) and Prof Madeleine Durbeej-Hjalt (Professor and Vice Chair of the Department of Experimental Medical Science at Lund University, Sweden). Professor Dubowitz (Emeritus Professor of Paediatrics, Imperial College London; and President of World Muscle Society) is our honorary scientific patron. To the extent that any member of the Scientific Advisory Board has any interest in the receipt of a grant he/she is excluded from the review and recommendation to the Trustees.

In order to ensure the DRF is exploring every avenue that could help Duchenne sufferers survive, we are continuously seeking to expand our research portfolio, as and when recommended by the Scientific Advisory Board, and we are anxious for scientists to submit relevant research proposals for our Scientific Advisory Board to consider. The charity has devised a robust two-tiered review process for any projects that it chooses to consider supporting.

At the DRF we aim to fund research related to the pathogenesis and treatment of Duchenne that includes minority mutations. We are highly committed to supporting the most promising research proposals that aim to find therapies and a cure. Following an informative summit between the trustees, founders and staff of the DRF and Dr Cohn in September 2017 to evaluate the current and future Duchenne research landscape, we identified several new avenues for funding in 2018. A key area of focus is to ensure more patients will be eligible to receive gene therapy treatments. We are therefore funding investigations into non-viral gene delivery methods (Project 1), and an immunological strategy to help manage patients who may have a pre-existing immunity to viral delivery methods (Project 2). By investing in both these approaches it is hoped that many more patients will have access to treatments. We are also funding next-generation genome editing (Project 3) and exploring options for improving outcome measures so that gene therapy treatments can be delivered as efficiently and safely as possible (Project 4).

In order to ensure new Duchenne treatments are successful in moving out of the laboratory and into clinical trial as quickly and safely as possible, the DRF continues to provide grants for clinical and administrative posts to help accelerate the clinical trial pipeline for Duchenne muscular dystrophy. The DRF also continues to nurture and sponsor emerging talent in the Duchenne research field to ensure the pipeline of science and treatments continues to evolve.

Please see our website www.duchenne.org.uk for further details on how to apply for a grant or contact research@duchenne.org.uk

REPORT OF THE TRUSTEES (CONTINUED)

Some of the research projects that the Duchenne Research Fund has funded in 2019 include:

PROJECT 1: Management of AAV Pre-Immunity for the treatment of Duchenne

Current AAV-mediated gene therapy trials exclude patients who have a pre-existing immunity to AAV – around one third of patients. In 2019 DRF funded a research programme at the University of Florida to develop a strategy to manage patients who have such a pre-existing immunity. The aim is to establish what level of immunity impacts on the safety and efficacy of AAV administration, and then focus on how best to modulate the immune system to allow for successful AAV administration. Overcoming this limitation in access to therapy will have a major impact in the field.

PROJECT 2: Next Generation Gene Therapy: Non-Viral Gene Delivery

At the end of 2018 DRF approved funding of Solid Biosciences' non-viral delivery research into alternative ways to deliver gene therapy to Duchenne patients, with the aim of increasing the number of patients who can receive gene therapy treatments.

Several clinical trials are now under way to explore the efficacy of gene transfer, a type of gene therapy, as a treatment for Duchenne. This entails using a micro-dystrophin – a shortened form of the dystrophin gene – with the aim of producing a functional dystrophin protein that patients' bodies can utilise. Current investigational gene transfer candidates for Duchenne make use of an adeno-associated virus (AAV). An AAV is a virus that is not known to cause disease and that acts as the delivery vehicle to carry the micro-dystrophin to patients' cells.

As viral-based gene therapies continue to demonstrate therapeutic benefit in the clinic, DRF believes it is crucial to also invest in non-viral delivery methods. These technologies may provide strategies to complement current viral gene therapies by potentially widening patient eligibility and offering methods to re-administer gene therapies to patients who have already received a viral-based therapy.

In 2019 the first stage of the project was under way, with Solid partnering with experts in nanoparticle formation and exosome-based delivery to develop and optimise non-viral delivery systems, as potential alternatives to AAV.

PROJECT 3: CRISPR for Duplications



In 2019 DRF funded the second year tranche of the grant awarded to the team at the Hospital for Sick Children in Toronto, Canada ('Sick Kids'), who are working on using CRISPR/Cas9 technology to correct duplication mutations for Duchenne. Dr Ronald Cohn had already had significant success as the first researcher to correct duplications in human cells in the lab, using CRISPR technology. His team have since been working to achieve the same results in mice that have Duchenne duplications, and have so far seen exciting results.

Recent advances in genome editing technologies based on CRISPR/Cas9 technology have the potential to correct the dystrophin gene, returning it to the correct full-length version of the gene. Rather than 'sending in' a shortened version of the gene, this more recent approach aims to correct just the mutated part of the gene. In the case of patients whose mutation is a duplication (where a part of the genetic code is repeated twice) the CRISPR approach entails sending in 'genetic scissors' into the cells to 'cut out' the duplicated part of the gene, restoring the correct full-length dystrophin. The aim is to translate this into boys and young men with Duchenne in the future.

In Q3 2019, at the end of the first year of the project, Dr Cohn's team reported that they had successfully created a new mouse model with the relevant genetic duplication in order to proceed to testing the CRISPR/Cas9 approach in vivo. Following the delivery of CRISPR the mice showed increased dystrophin expression and an improved performance.

REPORT OF THE TRUSTEES (CONTINUED)

PROJECT 4: 'KineDMD' extension

In 2017/18 DRF awarded a grant Dr Aldo Faisal's team at Imperial College London to develop a novel biomarker to track the progression of Duchenne. The study is called 'KineDMD'. As part of the study, both ambulant and non-ambulant Duchenne patients wear sensor devices on each wrist and ankle to monitor their daily activities and movements outside a clinical setting. The aim is for this approach to complement or even replace classic clinical endpoints, more sensitively capturing functional changes, and increasing the number of boys eligible for clinical trials. Following successful recruitment of patients in the first round and promising early data captured and analysed from the sensor devices, in Q2 2019 DRF agreed to fund an extension of the study to include more patients for a longer period up to 2021, and to gather more extensive data to be analysed. This study is currently being carried out as a clinical natural history study at the Clinical Research Facility of Great Ormond Street Hospital Trust, overseen by Professor Thomas Voit and Dr Valeria Ricotti.



PROJECT 5: Non-ambulant physiotherapy provision at Great Ormond Street Hospital

DRF had been aware for several years that there was a distinct lack of physiotherapy provision for boys and young men with Duchenne at Great Ormond Street Hospital (GOSH) once they were no longer ambulant (able to walk). Despite loss of ambulation, physiotherapy remains of enormous importance and benefit for upper body function in these patients. In 2016 DRF proposed to support a dedicated non-ambulant physiotherapy clinic at GOSH, however such a clinic required a business case from medical consultants and a financial commitment at the hospital Trust level as a consultant would need to be allocated to oversee the clinic.

DRF is pleased that after years of lobbying by the charity together with key clinicians, at GOSH significant progress had finally been made in 2019 towards establishing a clinic, and DRF therefore committed to fund a physiotherapist to fulfil this vital role. In Q3 2019 a physiotherapist already working within the Trust was identified to extend their role to cover the new care provision. It was anticipated that an agreement would be signed and the funds administered in Q1 2020.



PROJECT 6: Newcastle University Clinical Research Fellow

The DRF and a consortium of seven UK Duchenne charities continue to fund a Fellowship for Dr Michela Guglieri in partnership with Newcastle University and Newcastle University Teaching Hospital, to develop and advance the clinical trial agenda for Duchenne muscular dystrophy. The award of £250,000 over five years is being matched by Newcastle University to enable Dr Guglieri to develop the clinical trial capacity for Duchenne at the John Walton Muscular Dystrophy Research Centre and nationally.

PROJECT 7: TREAT-NMD Programme Coordinator

The DRF jointly funds a programme coordinator post at the John Walton Muscular Dystrophy Research Centre at Newcastle University. The post is held by Cathy Turner, who has worked with funders and key stakeholders to ensure the needs and input of the Duchenne community are addressed during the research and clinical process. The role is being funded by DRF together with Duchenne UK and Joining Jack.



REPORT OF THE TRUSTEES (CONTINUED)

PROJECT 8: International World Muscle Society Congress – Duchenne Research Prize

The DRF continues to fund the Duchenne Research Prize for an up-and-coming researcher at the International World Muscle Society's annual symposium. The winner at the 24th congress in October 2019 in Copenhagen was Dr Russell Rogers, whose post-doctoral research investigates whether cardiac progenitor cells could be beneficial in preventing the onset of Duchenne or delaying disease progression.



[Read more about our projects at www.duchenne.org.uk/our-projects](http://www.duchenne.org.uk/our-projects)

PUBLIC BENEFIT

The Trustees confirm that they have referred to the guidance contained in the Charity Commission's general guidance on public benefit when reviewing the Fund's aim and objectives and in planning future activities and setting the grant making policy for the year.

FINANCIAL REVIEW

Total income for the year was £373,050 (2018: £1,871,935). Total expenditure for the year was £548,265 (2018: £603,636). Of this, £467,720 (2018: £532,897) was provided as grants payable towards research into Duchenne muscular dystrophy. The result for the year was a loss of £175,215 before gains and losses (2018: surplus £1,268,299).

The unrestricted funds at 31 December 2019 totalled £2,532,754 (2018: £5,770,562). Restricted funds totalled £0 at 31 December 2019 (2018: £520).

RESERVES POLICY

On an ongoing basis the Trustees aim to hold minimal reserves as the general cost base is low and before commencing any project the Trustees aim to raise the majority of the required income pertaining to each project. However the trustees are conscious that new development and research is dynamic and projects worthy of funding may arise at any time and believe it prudent to maintain some reserve for such eventuality. Considerable reserves are currently being held due to the sale of shares in Solid Biosciences in 2018 and the Trustees and Scientific Advisory Board are evaluating and considering additional research projects on an ongoing basis. The reserve policy will be reviewed on an annual basis.

INVESTMENT PERFORMANCE

The Trust Deed authorises the Trustees to make and hold investments, using the general funds of the charity. The Duchenne Research Fund were extremely fortunate to have surplus funds due to the sale of shares in Solid Biosciences by DRIL in 2018 which the Duchenne Research Fund has a third shareholding in. The Trustees felt it was prudent to revisit the charity's investment strategy.

The Trustees use a current account with limited funds held to cover running costs. Additional funds are held in a deposit account which is accessible, and this is used to cover medical research and other charitable expenditure as projects arise and are agreed upon as suitable by the Trustees. However whilst considering future projects the Trustees felt that due to the current low rates of interest the funds held in the deposit account were generating very low returns. After much consideration of different options, the Trustees decided to invest a million pounds with Delmore Asset Management in the hope that greater returns could be achieved for the charity. Delmore Asset Management receive fees for managing the funds.

REPORT OF THE TRUSTEES (CONTINUED)

POST BALANCE SHEET EVENTS

The advent of the Covid-19 pandemic has impacted the DRF's operations. There is less work for DRF staff to undertake in 2020, as the closure of universities and the circumstances of clinicians working against the backdrop of the crisis means it is unlikely that many new research projects will be reviewed and agreed in 2020. It is also not possible to hold any in-person donor or fundraising events at this time. The decision has been taken to furlough the Donor & Fundraising Manager. The charity will cover 20% of the employee's salary to make up the shortfall from the government furlough scheme.

Ongoing support, correspondence and tranche payments for existing grantees; administration of grants that were close to being agreed before the start of the pandemic; receipt and acknowledgement of donations and individual fundraising efforts; and general administrative work have all continued as before.

Following government guidance to work from home wherever possible, the Director of Operations is working from home. Due to schools being closed or running reduced provision, work will necessarily be carried out on a flexible basis. Staff arrangements will be reviewed as and when government guidance changes. In the event that employee(s) need to attend the office while the pandemic is ongoing, a risk assessment will be undertaken, social distancing will be adhered to as much as possible, and a cleaning schedule will be implemented.

The financial statements have been prepared on a going concern basis as the charity has sufficient funds to cover running costs for the next 12 months and all existing research commitments.

Fees that have already been paid for participation in external fundraising events will either be refunded or carried over to 2021. In the case of the Prudential RideLondon-Surrey, the 10 places already paid for will be converted to five London Marathon places for the 2021 race. The race has been postponed from April to October 2021.

STRUCTURE, GOVERNANCE AND MANAGEMENT

Constitution

The Charity is an unincorporated entity, governed by a Declaration of Trust dated 25 March 2007. It is a registered charity, number 1119068. The charity received charitable status on 3 May 2007 and started operating on 17 May 2007.

Trustees

The following Trustees have served during the year to date:

Daniel Baum
Simon Kanter
Sarah Shelley
Jeremy Shebson

At all times there shall be at least three Trustees holding office. Under the provisions of the Trust Deed, new Trustees are appointed by a resolution of the Trustees passed at a special meeting. The Board of Trustees is authorised to appoint new Trustees to fill vacancies arising because of resignation or the demise of an existing Trustee.

REPORT OF THE TRUSTEES (CONTINUED)

STATEMENT OF TRUSTEES' RESPONSIBILITIES

The Trustees are responsible for preparing the Trustees' Annual Report and the financial statements in accordance with applicable law and United Kingdom Accounting Standards (United Kingdom Generally Accepted Accounting Practice).

The law applicable to charities in England & Wales requires the Trustees to prepare financial statements for each financial year which give a true and fair view of the state of affairs of the charity and of the incoming resources and application of resources 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 in the Charities SORP 2015 (FRS 102);
- make judgements and estimates that are reasonable and prudent;
- state whether applicable accounting standards have been followed, subject to any material departures disclosed and explained in the financial statements;
- prepare the financial statements on the going concern basis unless it is inappropriate to presume that the charity will continue in operation.

The Trustees are responsible for keeping proper accounting records that 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 Charities Act 2011, the Charity (Accounts and Reports) Regulations 2008 and the provisions of the trust deed. They are also responsible for safeguarding the assets of the charity and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

Approved by the Trustees and signed on their behalf:

Simon Kanter – Chairman
Trustee

Date: 16/10/20

INDEPENDENT AUDITOR'S REPORT TO THE TRUSTEES

OPINION

We have audited the financial statements of The Duchenne Research Fund (the 'charity') for the year ended 31 December 2019 which comprise the Statement of Financial Activities, Balance Sheet, Statement of Cash Flows and notes to the financial statements, including a summary of significant accounting policies. The financial reporting framework that has been applied in their preparation is applicable law and United Kingdom Accounting Standards, including Financial Reporting Standard 102 'The Financial Reporting Standard applicable in the UK (United Kingdom Generally Accepted Accounting Practice).

In our opinion, the financial statements:

- give a true and fair view of the state of the charity's affairs as at 31 December 2019 and of its incoming resources and application of resources, for the year then ended;
- have been properly prepared in accordance with United Kingdom Generally Accepted Accounting Practice; and
- have been prepared in accordance with the requirements of the Charities Act 2011.

BASIS FOR OPINION

We conducted our audit in accordance with International Standards on Auditing (UK) (ISAs (UK)) and applicable law. Our responsibilities under those standards are further described in the Auditor's responsibilities for the audit of the financial statements section of our report. We are independent of the charity in accordance with the ethical requirements that are relevant to our audit of the financial statements in the UK, including the FRC's Ethical Standard, and we have fulfilled our other ethical responsibilities in accordance with these requirements. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.

CONCLUSIONS RELATING TO GOING CONCERN

We have nothing to report in respect of the following matters in relation to which the ISAs (UK) require us to report to you where:

- the trustees' use of the going concern basis of accounting in the preparation of the financial statements is not appropriate; or
- the trustees have not disclosed in the financial statements any identified material uncertainties that may cast significant doubt about the charity's ability to continue to adopt the going concern basis of accounting for a period of at least twelve months from the date when the financial statements are authorised for issue.

OTHER INFORMATION

The trustees are responsible for the other information. The other information comprises the information included in the annual report, other than the financial statements and our auditor's report thereon. Our opinion on the financial statements does not cover the other information and we do not express any form of assurance conclusion thereon.

In connection with our audit of the financial statements, our responsibility is to read the other information and, in doing so, consider whether the other information is materially inconsistent with the financial statements or our knowledge obtained in the audit or otherwise appears to be materially misstated. If we identify such material inconsistencies or apparent material misstatements, we are required to determine whether there is a material misstatement in the financial statements or a material misstatement of the other information. If, based on the work we have performed, we conclude that there is a material misstatement of this other information, we are required to report that fact.

We have nothing to report in this regard.

MATTERS ON WHICH WE ARE REQUIRED TO REPORT BY EXCEPTION

We have nothing to report in respect of the following matters in relation to which the Charities (Accounts and Reports) Regulations 2008 requires us to report to you if, in our opinion:

- the information given in the financial statements is inconsistent in any material respect with the trustees' report; or
- sufficient accounting records have not been kept; or
- the financial statements are not in agreement with the accounting records; or
- we have not received all the information and explanations we require for our audit.

INDEPENDENT AUDITOR'S REPORT TO THE TRUSTEES (CONTINUED)

RESPONSIBILITIES OF TRUSTEES

As explained more fully in the trustees' responsibilities statement, the trustees are responsible for the preparation of the financial statements and for being satisfied that they give a true and fair view, and for such internal control as the trustees determine is necessary to enable the preparation of financial statements that are free from material misstatement, whether due to fraud or error.

In preparing the financial statements, the trustees are responsible for assessing the charity's ability to continue as a going concern, disclosing, as applicable, matters related to going concern and using the going concern basis of accounting unless the trustees either intend to liquidate the charity or to cease operations, or have no realistic alternative but to do so.

AUDITOR'S RESPONSIBILITIES FOR THE AUDIT OF THE FINANCIAL STATEMENTS

We have been appointed as auditor under section 144 of the Charities Act 2011 and report in accordance with the Act and relevant regulations made or having effect thereunder.

Our objectives are to obtain reasonable assurance about whether the financial statements as a whole are free from material misstatement, whether due to fraud or error, and to issue an auditor's report that includes our opinion. Reasonable assurance is a high level of assurance, but is not a guarantee that an audit conducted in accordance with ISAs (UK) will always detect a material misstatement when it exists. Misstatements can arise from fraud or error and are considered material if, individually or in the aggregate, they could reasonably be expected to influence the economic decisions of users taken on the basis of these financial statements.

A further description of our responsibilities for the audit of the financial statements is located on the Financial Reporting Council's website at www.frc.org.uk/auditorsresponsibilities. This description forms part of our auditor's report.

USE OF OUR REPORT

This report is made solely to the charity's trustees, as a body, in accordance with Part 4 of the Charities (Accounts and Reports) Regulations 2008. Our audit work has been undertaken so that we might state to the charity's trustees those matters we are required to state to them in an auditor's report and for no other purpose. To the fullest extent permitted by law, we do not accept or assume responsibility to anyone other than the charity and the charity's trustees as a body, for our audit work, for this report, or for the opinions we have formed.

Michelle Wilkes (Senior Statutory Auditor)
For and on behalf of Azets Audit Services
Statutory Auditor
Greytown House
221-227 High Street
Orpington BR6 0NZ

16/10/20

STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2019

	Notes	Unrestricted funds £	Restricted funds £	Total 2019 £	Total 2018 £
INCOME FROM:					
Donations	2	50,278	-	50,278	85,779
Investment income	3	321,572	-	321,572	1,785,456
Other	4	1,200	-	1,200	700
Total income		373,050	-	373,050	1,871,935
EXPENDITURE ON:					
Raising funds	6	13,838	-	13,838	3,693
Charitable activities	7	533,907	520	534,427	599,943
Total expenditure	5	547,745	520	548,265	603,636
Net expenditure/income		(174,695)	(520)	(175,215)	1,268,299
Loss on investments	12,13	(3,063,113)	-	(3,063,113)	2,930,937
Net movements in funds		(3,237,808)	(520)	(3,238,328)	4,199,236
Fund balances brought forward 1 January 2019		5,770,562	520	5,771,082	1,571,846
Fund balance carried forward 31 December 2019		2,532,754	-	2,532,754	5,771,082

► All transactions are derived from continuing activities.

STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2019

COMPARATIVE 31 DECEMBER 2018		Unrestricted funds	Restricted funds	Total 2018
	Notes	£	£	£
INCOME FROM:				
Donations	2	80,219	5,560	85,779
Investment income		1,785,456	-	1,785,456
Other	3	700	-	700
Total income		1,866,375	5,560	1,871,935
EXPENDITURE ON:				
Raising funds	6	3,693	-	3,693
Charitable activities	7	594,903	5,040	599,943
Total expenditure	5	598,596	5,040	603,636
Net expenditure/income		1,267,779	520	1,268,299
Gains on investments	13	2,930,937	-	2,930,937
Net movements in funds		4,198,716	520	4,199,236
Fund balances brought forward 1 January 2018		1,571,846	-	1,571,846
Fund balance carried forward 31 December 2018		5,770,562	520	5,771,082

► All transactions are derived from continuing activities.

BALANCE SHEET AS AT 31 DECEMBER 2019

	Notes	2019 £	2018 £
Fixed assets			
Tangible fixed assets	11	-	-
Investments	12	996,942	-
Programme Related Investments	13	581,794	3,643,399
		1,578,736	3,643,399
Current assets			
Debtors	14	3,015	1,213
Cash at bank and in hand		979,506	2,652,486
		982,521	2,653,699
Creditors: Amounts falling due within one year	15	(28,503)	(526,016)
Net current assets		954,018	2,127,683
Net assets		2,532,754	5,771,082
FUNDS			
Restricted funds	16	-	520
Unrestricted general funds		2,532,754	5,770,562
	18	2,532,754	5,771,082

The financial statements were approved on 16/10/20 and signed on behalf of the Board by:

Simon Kanter - Chairman
Trustee

Daniel Baum - Treasurer
Trustee

STATEMENT OF CASH FLOWS AS AT 31 DECEMBER 2019

	Notes	2019 £	2018 £
Cash flow from operating activities	19	(996,102)	380,637
Net cash flow from operating activities		(996,102)	380,637
Cash flow from investing activities			
Interest received		884	813
Programme Related Investment dividends receivable		315,338	1,784,643
Purchase of investments		(1,000,000)	-
Investment manager fees		6,900	-
Net cash flow from investing activities		(676,878)	1,785,456
Net (decrease)/ increase in cash and cash equivalents		(1,672,980)	2,166,093
Cash and cash equivalents at 01 January 2019		2,652,486	486,393
Cash and cash equivalents at 31 December 2019		979,506	2,652,486
Cash and cash equivalents consists of:			
Cash at bank and in hand		979,506	2,652,486
Cash and cash equivalent at 31 December 2019		979,506	2,652,486

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

1. ACCOUNTING POLICIES

1.1 Basis of preparation

The Duchenne Research Fund is a company limited by guarantee in the United Kingdom. In the event of the charity being wound up, the liability in respect of the guarantee is limited to £1 per member of the charity. The address of the registered office is given in the charity information on page 1 of these financial statements. The nature of the charity's operations and principal activities are set out on page 2.

The charity constitutes a public benefit entity as defined by FRS 102. The financial statements have been prepared in accordance with Accounting and Reporting by Charities: 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) issued on 16 July 2014, the Charities Act 2011 and UK Generally Accepted Practice as it applies from 1 January 2015.

The financial statements are prepared on a going concern basis under the historical cost convention, modified to include certain items at fair value. The financial statements are prepared in sterling which is the functional currency of the charity.

The significant accounting policies applied in the preparation of these financial statements are set out below. These policies have been consistently applied to all years presented unless otherwise stated.

1.2 Income

All income is included in the Statement of Financial Activities (SoFA) when the charity is legally entitled to the income after any performance conditions have been met, the amount can be measured reliably and it is probable that the income will be received.

For donations to be recognised the charity will have been notified of the amounts and the settlement date in writing. If there are conditions attached to the donation and this requires a level of performance before entitlement can be obtained then income is deferred until those conditions are fully met or the fulfilment of those conditions is within the control of the charity and it is probable that they will be fulfilled.

Investment income is earned through holding assets for investment purposes such as shares. It includes dividends and interest. Where it is not practicable to identify investment management costs incurred within a scheme with reasonable accuracy the investment income is reported net of these costs. It is included when the amount can be measured reliably. Interest income is recognised using the effective interest method and dividend and rent income is recognised as the charity's right to receive payment is established.

1.3 Expenditure

All expenditure is accounted for on an accruals basis and has been classified under headings that aggregate all costs related to the category. Expenditure is recognised where there is a legal or constructive obligation to make payments to third parties, it is probable that the settlement will be required and the amount of the obligation can be measured reliably. It is categorised under the following headings:

- Costs of raising funds includes those costs incurred by the charity when marketing and fundraising to raise fund for the charity; and
- Expenditure on charitable activities comprises those costs incurred by the charity in the delivery of its activities and services for its beneficiaries. It includes both costs allocated directly to such activities and those costs of an indirect nature necessary to support them.

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

1.4 Support costs allocation

Support costs are those that assist the work of the charity but do not directly represent charitable activities and include office costs, governance costs, administrative payroll costs. They are incurred directly in support of expenditure on the objects of the charity and include project management carried out at Headquarters. Where support costs cannot be directly attributed to particular headings they have been allocated to cost of raising funds and expenditure on charitable activities on a basis consistent with use of the resources.

Fund-raising costs are those incurred in seeking voluntary contributions and do not include the costs of disseminating information in support of the charitable activities.

The analysis of these costs is included in note 6.

1.5 Funds

Unrestricted funds are available for use at the discretion of the trustees in furtherance of the general objectives of the charity and which have not been designated for other purposes.

Restricted funds – these are funds that can only be used for specific restricted purposes within the objects of the charity. Restrictions arise when specified by the donor or when funds are raised for particular restricted purposes.

1.6 Investments

Investments are recognised initially at their transaction value, which is normally the transaction price less transaction costs. Subsequently, they are measured at fair value with changes recognised in ‘net gains / (losses) on investments’ in the SoFA if the shares are publicly traded or their fair value can otherwise be measured reliably.

1.7 Debtors and creditors receivable / payable within one year

Debtors and creditors with no stated interest rate and receivable or payable within one year are recorded at transaction price. Any losses arising from impairment are recognised in expenditure.

1.8 Loans

Loans are initially recognised at the transaction price including transaction costs. Subsequently, they are measured at amortised cost using the effective interest rate method, less impairment. If an arrangement constitutes a finance transaction it is measured at present value.

1.9 Foreign currencies

Assets and liabilities in foreign currencies are translated into Sterling at the rates of exchange ruling at the balance sheet date. Transactions in foreign currencies are translated into Sterling at the rate of exchange ruling at the date of transaction. Exchange differences are taken into account in arriving at the operating result.

1.10 Tangible fixed assets

Tangible fixed assets are stated at cost (or deemed cost) or valuation less accumulated depreciation and accumulated impairment losses. Assets costing less than £500 are written off in the period of acquisition. All other assets are capitalised.

1.11 Depreciation

Depreciation is provided on a straight line basis on the cost of tangible fixed assets, to write them down to their estimated residual values over their expected useful lives. Assets are depreciated over 4 years but this commences the year after acquisition.

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

1.12 Going concern

The financial statements have been prepared on a going concern basis as the trustees believe that no material uncertainties exist. The trustees have considered the level of funds held and the expected level of income and expenditure for 12 months from authorising these financial statements. The budgeted income and expenditure is sufficient with the level of reserves for the charity to be able to continue as a going concern. This included consideration of the effect of the Covid-19 virus on the charity's operations.

1.13 Judgements and key sources of estimation uncertainty

Accounting estimates and judgements are continually evaluated and are based on historical experience and other factors, including expectations of future events that are believed to be reasonable under the circumstances.

The following judgements (apart from those involving estimates) have been made in the process of applying the above accounting policies that have had the most significant effect on amounts recognised in the financial statements:

Useful economic lives of tangible assets

The annual depreciation charge for tangible assets is sensitive to changes in the estimated useful economic lives and residual values of the assets. The useful economic lives and residual values are re-assessed annually. They are amended when necessary to reflect current estimates, based on technological advancement, future investments, economic utilisation and the physical condition of the assets. See note 10 for the carrying amount of the property plant and equipment, and note 1.10 for the useful economic lives for each class of assets.

There are no key assumptions concerning the future and other key sources of estimation uncertainty at the reporting date that have a significant risk of causing a material adjustment to the carrying amounts of assets and liabilities within the next financial year.

2. DONATIONS

	2019 £	2018 £
Restricted funds:		
Donations	-	5,560
Unrestricted funds:		
Donations	32,401	34,506
Dinner income 2016	-	3,250
Yorkshire Three Peaks Challenge	10,000	10,000
Prudential RideLondon-Surrey	7,877	32,463
	50,278	85,779

3. INVESTMENT INCOME

	2019 £	2018 £
Interest	884	813
Programme Related Investment dividend receivable	315,338	1,784,643
Investment dividends	5,350	-
	321,572	1,785,456

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

4. OTHER INCOME	2019 £	2018 £
Unrestricted funds:		
Rental income	1,200	700
	1,200	700

5. ANALYSIS OF EXPENDITURE	Staff costs £	Direct costs £	Support costs £	Total 2019 £	Total 2018 £
Raising funds	-	13,838	-	13,838	3,693
Charitable activities	41,017	467,720	25,690	534,427	599,943
Total expenditure	41,017	481,558	25,690	548,265	603,636

Support costs have all been allocated against charitable activities.

6. RAISING FUNDS	2019 £	2018 £
Website marketing	627	114
Prudential Bike Ride	2,814	2,700
Investment manager fee	6,900	-
Other costs	3,497	879
	13,838	3,693

7. DIRECT CHARITABLE EXPENDITURE	2019 £	2018 £
Restricted expenditure:		
Solid – Non-viral delivery research	-	5,040
KineDMD – Imperial College London	520	-
Unrestricted expenditure:		
KineDMD – Imperial College London	233,191	-
Treat-NMD Programme Co-ordinator	15,328	15,445
Management of AAV Pre-Immunity - University of Florida	84,552	-
Newcastle University Clinical Research Fellowship (administered by Action Duchenne)	7,143	7,143
World Muscle Society	500	500
Gene therapy for Becker – UCL	27,047	81,142
CRISPR for Duplications – Hospital for Sick Children, Toronto	99,439	108,667
Solid – Non-viral delivery research	-	314,960
	467,720	532,897

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

8. SUPPORT COSTS	2019 £	2018 £
Legal costs	362	573
Bank charges	712	735
Postage, stationery and telephone	184	238
Administration	2,776	2,975
Rent and service costs	7,122	8,288
Travel costs	2,002	559
Insurance	2,182	2,197
Foreign exchange variance	4,802	-
Other	388	846
Governance	5,160	4,464

	25,690	20,875
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9. GOVERNANCE COSTS	2019 £	2018 £
Auditors' remuneration - current year	4,440	4,320
- under accrual in prior year	720	144
	5,160	4,464

10. STAFF COSTS AND TRUSTEE REMUNERATION	2019 £	2018 £
Wages and salaries	39,577	44,821
Social Security costs	534	776
Pension Costs	906	574
	41,017	46,171

Two members of staff (head count) were employed during 2019 (2018: Two). No employees earned over £60,000 per annum.

No benefits are received by key management personnel (2018: £Nil). The Trust considers its key management personnel to comprise those individuals listed on page 1.

None of the Trustees received any remuneration or reimbursed expenses during the year. During the year £1,987 (2018: £1,595) was paid for Trustee Indemnity Insurance.

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

11. TANGIBLE FIXED ASSETS

Furniture and
equipment
£

Cost:

At 1 January 2019 & 31 December 2019

3,547

Depreciation:

At 1 January 2019 & 31 December 2019

3,547

Net book value:

At 1 January 2019 & 31 December 2019

-

12. FIXED ASSET INVESTMENTS

2019
£

2018
£

Market value at 1 January 2019

-

-

Additions

1,000,000

-

Reinvested income

5,350

-

Costs

(6,900)

-

Change in value in the year

(1,508)

-

Market value at 31 December 2019

996,942

-

Historic cost at 31 December 2019

1,000,000

-

The fair value of listed investments is determined by reference to the mid-market value of one share in the Fund at the Balance Sheet date.

This balance is made up of:

Debt and fixed interest

327,232

-

Equities

266,762

-

Infrastructure

177,462

-

Cash

225,486

-

996,942

-

The following investments are significant holdings within the investment portfolio:

Ishares II PLC CORE UK Gilts

49,666

-

Ishares III PLC UK Gilts

50,057

-

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

13. PROGRAMME RELATED INVESTMENTS

	2019 £	2018 £
Market value of investment at 1 January 2019	3,643,399	712,462
(Loss)/gains on investment	(3,061,605)	2,930,937
Market value at 31 December 2019	581,794	3,643,399
Historic cost at 31 December 2019	712,462	712,462

Duchenne Research Fund is a shareholder in Duchenne Research (UK) Investment Ltd (“DRIL”), a company incorporated in the UK as a company limited by shares on 9 December 2014.

The purpose of DRIL is to invest in SOLID GT LLC, a limited liability corporation incorporated on 5 August 2014 in Delaware, USA.

The Trustees hold the investment in SOLID GT LLC as part of their long term strategy. The market value at the year end will be dependent upon prevailing market conditions and is taken at a fixed date. The Trustees do not consider this to be a permanent diminution in value.

The purpose of SOLID GT LLC is to undertake gene therapy-based research in Duchenne muscular dystrophy and drug development and treatments in connection therewith. During March 2017 SOLID GT LLC merged with Solid Biosciences Inc. On 26 January 2018 Solid Biosciences was listed on the Nasdaq and the Common C Stock was converted to 520,477 ordinary shares at a conversion rate of 0.8485 per C stock.

The charity’s investment in DRIL has been adjusted to its share (one third) of the value Solid Biosciences shares held by DRIL at 31 December 2019.

During the year DRIL declared a dividend to Duchenne Research Fund of £315,338 (2018: £1,784,643).

One director of DRIL, Sarah Shelley, was a trustee of Duchenne Research Fund during the year.

14. DEBTORS

	2019 £	2018 £
Gift Aid receivable	944	1,213
Other debtors	2,071	-
	3,015	1,213

15. CREDITORS Amounts falling due within one year

	2019 £	2018 £
Accruals	5,032	6,456
Other taxes and social security	-	2,108
Grants payable	23,471	517,452
	28,503	526,016

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

16. RESTRICTED FUNDS

	Balance at 1.01.19 £	Income £	Expenditure £	Balance at 31.12.19 £
2019				
Research grants	520	-	(520)	-
	520	-	(520)	-

	Balance at 1.01.18 £	Income £	Expenditure £	Balance at 31.12.18 £
2018				
Research grants	-	5,560	(5,040)	520
	-	5,560	(5,040)	520

17. RELATED PARTY TRANSACTIONS

One trustee, Sarah Shelley, was a director of Duchenne Research (UK) Investment Ltd ("DRIL"). During the year, dividends totalling £315,338 were receivable (2018: £1,784,643).

18. ANALYSIS OF NET ASSETS BETWEEN FUNDS

	Restricted 2019 £	Unrestricted 2019 £	Total 2019 £
2019			
Fixed assets	-	1,578,736	1,578,736
Current assets	-	982,521	982,521
Current liabilities	-	(28,503)	(28,503)
	-	2,532,754	2,532,754

	Restricted 2018 £	Unrestricted 2018 £	Total 2018 £
2018			
Fixed assets	-	3,643,399	3,643,399
Current assets	520	2,653,179	2,653,699
Current liabilities	-	(526,016)	(526,016)
	520	5,770,562	5,771,082

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2019

19. RECONCILIATION OF NET (EXPENDITURE) / INCOME TO NET CASH FLOW FROM OPERATING ACTIVITIES	2019 £	2018 £
Net (expenditure) / income for 31 December 2019	(175,215)	1,268,299
Interest received	(884)	(813)
Dividends received	(320,688)	(1,784,643)
(Increase) / decrease in debtors	(1,802)	381,853
(Decrease) / increase in creditors	(497,513)	515,941
Net cash flow from operating activities	(996,102)	380,637

20. POST BALANCE SHEET EVENT

Subject to the financial impact of Covid-19 that has arisen in March 2020, the Trustees have assessed the operational and financial impact on the charity in the Report of the Trustees on page 7 and in the going concern statement at note 1.12.