



# Duchenne Research Fund

**THE DUCHENNE RESEARCH FUND**

**REPORT AND FINANCIAL STATEMENTS  
FOR THE YEAR ENDED 31 DECEMBER 2018**

**Registered Charity No. 1119068**

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**WILKINS KENNEDY 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 this devastating disease. Help us find a cure.*



*#TeamDRF cyclists raised nearly £35,000 for Duchenne research at the Prudential RideLondon-Surrey cycle ride on 29 July 2018*

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## REFERENCE AND ADMINISTRATIVE DETAILS

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|-----------------------------|--|
| Trustees                    | Daniel Baum<br>Simon Kanter<br>Jeremy Shebson<br>Sarah Shelley   |
| Charity Registration Number | 1119068  |
| Principal Office            | Symal House<br>423 Edgware Road<br>London<br>NW9 0HU   |
| Key Management              | Trustees   |
| Banker                      | Bank of Scotland<br>Teviot House<br>41 South Gyle Crescent<br>Edinburgh<br>EH12 9DR  |
| Statutory Auditor           | Wilkins Kennedy Audit Services<br>Statutory Auditor<br>Greytown House<br>221 – 227 High Street<br>Orpington<br>Kent<br>BR6 0NZ |



## REPORT OF THE TRUSTEES

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The Trustees present their report and audited financial statements for the year ended 31 December 2018.

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](http://www.duchenne.org.uk)**

### FUNDRAISING IN 2018

#### PRUDENTIAL RideLondon-Surrey

Following the success of the Yorkshire Three Peaks event in 2017 several supporters expressed interest in a summer 2018 sporting challenge. The DRF applied to take places at the Prudential RideLondon-Surrey 100 mile cycling event on 29 July 2018. In early 2018 nine cyclists signed up to take part in the ride and raise funds for DRF. We are extremely grateful to them and their supporters who raised a total of nearly £35,000.

#### OTHER FUNDRAISING ACTIVITY

The DRF is reliant on fundraising to sustain 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. Following the success of the Prudential RideLondon-Surrey cycle ride the DRF will take places at the event on 4 August 2019. Further information can be found at [www.duchenne.org.uk/cycle](http://www.duchenne.org.uk/cycle)

Please contact [info@duchenne.org.uk](mailto: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)

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### 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. It is anticipated that a balance of the funds from this sale of shares will be transferred to the charities (including the DRF) in 2019. The trustees and Scientific Advisory Board will continue to work together during 2019 to ensure these funds are put to the most effective use. 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 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](http://www.duchenne.org.uk/news), or sign up for our newsletter via the homepage.

### FUNDING STRATEGY IN 2018

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; Chief of Clinical and Metabolic Genetics and Co-director of the Centre for Genetic Medicine at the Hospital for Sick Children, Toronto, as well as incoming President and CEO of the hospital, effective 2019). 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](http://www.duchenne.org.uk) for further details on how to apply for a grant or contact [research@duchenne.org.uk](mailto:research@duchenne.org.uk)

## REPORT OF THE TRUSTEES (CONTINUED)

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Some of the research projects that the Duchenne Research Fund has funded in 2018 include:

### **PROJECT 1: Next Generation Gene Therapy: Non-Viral Gene Delivery**

In 2018 DRF agreed to fund 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.

DRF will fund Solid Biosciences' non-viral delivery research programme, which has partnered with experts in nanoparticle formation and exosome-based delivery to develop and optimise non-viral delivery systems, as potential alternatives to AAV.

### **PROJECT 2: Management of AAV Pre-Immunity for the treatment of Duchenne**

Current AAV-mediated gene therapy trials exclude subjects who have a pre-existing immunity to AAV – around one third of patients. In 2018 DRF agreed to fund research 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 3: CRISPR for Duplications**

In 2018 DRF awarded a grant 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.

## REPORT OF THE TRUSTEES (CONTINUED)

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### PROJECT 4: 'KineDMD' extension

In 2017 the 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 2017-18 and promising early data captured and analysed from the sensor devices, Dr Faisal proposed an extension of the study to include more patients for a longer period, and to gather more extensive data to be analysed. Following review by the SAB and Trustee Board, DRF plans to fund the extension in 2019. 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.

**Imperial College  
London**

### PROJECT 5: 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.

The Newcastle upon Tyne Hospitals   
NHS Foundation Trust



### PROJECT 6: 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.



### PROJECT 7: 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 23rd congress in October 2018 in Mendoza, Argentina, was Hesham Ismail Hamed from the University of Geneva



### PROJECT 8: The Duchenne Forum

The DRF continued to fund research projects as part of the Duchenne Forum, a collaboration between UK Duchenne charities to accelerate progress in the search for effective treatments and eventually cures. The partnership has supported nine pioneering Duchenne muscular dystrophy research projects, to which the Duchenne Forum has contributed £840,000 between 2013 and 2018.

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



## REPORT OF THE TRUSTEES (CONTINUED)

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### 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 £1,871,935 (2017: £596,437). Total expenditure for the year was £603,636 (2017: £725,386). Of this, £532,897 (2017: £335,340) was provided as grants payable towards research into Duchenne muscular dystrophy. The result for the year was a surplus of £1,268,299 before gains and losses (2017: deficit £128,949).

The unrestricted funds at 31 December 2018 totalled £5,770,562 (2017: £1,571,846). Restricted funds totalled £520 at 31 December 2018 (2017: £0).

### 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 sale of shares and fundraising, and the Trustees and Scientific Advisory Board are now evaluating and considering additional research projects. 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. From 2008 the surplus funds are placed in a high interest account and utilised for general purposes as a contingency fund as authorised by the Trustees.

### 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)

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### 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: 15/08/19

## INDEPENDENT AUDITOR'S REPORT TO THE TRUSTEES

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### OPINION

We have audited the financial statements of The Duchenne Research Fund (the 'charity') for the year ended 31 December 2018 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 2018 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)**

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### **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](http://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 Wilkins Kennedy Audit Services  
Statutory Auditor  
Greytown House  
221-227 High Street  
Orpington BR6 0NZ

21/08/19

## STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2018

|  | Notes    | Unrestricted<br>funds<br>£ | Restricted<br>funds<br>£ | Total<br>2018<br>£ | Total<br>2017<br>£ |
|--|----------|----------------------------|--------------------------|--------------------|--------------------|
| <b>INCOME FROM:</b>  |          |                            |                          |                    |                    |
| Donations  | <b>2</b> | 80,219                     | 5,560                    | 85,779             | 212,448            |
| Investment income  | <b>3</b> | 1,785,456                  | -                        | 1,785,456          | 378,989            |
| Other  | <b>4</b> | 700                        | -                        | 700                | 5,000              |
| <b>Total income</b>  |          | <b>1,866,375</b>           | <b>5,560</b>             | <b>1,871,935</b>   | <b>596,437</b>     |
| <b>EXPENDITURE ON:</b>   |          |                            |                          |                    |                    |
| Raising funds  | <b>5</b> | 3,693                      | -                        | 3,693              | 11,570             |
| Charitable activities  | <b>5</b> | 594,903                    | 5,040                    | 599,943            | 335,340            |
| Conversion of Programme<br>Related Investment to<br>distributable reserves |          | -                          | -                        | -                  | 378,476            |
| <b>Total expenditure</b>   | <b>5</b> | <b>598,596</b>             | <b>5,040</b>             | <b>603,636</b>     | <b>725,386</b>     |
| <b>Net movements in funds</b>  |          | <b>1,267,779</b>           | <b>520</b>               | <b>1,268,299</b>   | <b>(128,949)</b>   |
| <b>Gains on investments</b>  |          | <b>2,930,937</b>           | <b>-</b>                 | <b>2,930,937</b>   | <b>-</b>           |
| <b>Fund balances brought forward<br/>1 January 2018</b>                    |          | <b>1,571,846</b>           | <b>-</b>                 | <b>1,571,846</b>   | <b>1,700,795</b>   |
| <b>Fund balance carried forward<br/>31 December 2018</b>                   |          | <b>5,770,562</b>           | <b>520</b>               | <b>5,771,082</b>   | <b>1,571,846</b>   |

► All transactions are derived from continuing activities.



## STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2018

| COMPARATIVE<br>31 DECEMBER 2017  |          | Unrestricted<br>funds | Restricted funds | Total<br>2017    |
|--|----------|-----------------------|------------------|------------------|
|  | Notes    | £                     | £                | £                |
| <b>INCOME FROM:</b>  |          |                       |                  |                  |
| Donations  | 2        | 211,948               | 500              | 212,448          |
| Investment income  |          | 378,989               | -                | 378,989          |
| Other  | 3        | 5,000                 | -                | 5,000            |
| <b>Total income</b>  |          | <b>595,937</b>        | <b>500</b>       | <b>596,437</b>   |
| <b>EXPENDITURE ON:</b>   |          |                       |                  |                  |
| Raising funds  | 5        | 11,570                | -                | 11,570           |
| Charitable activities  | 5        | 304,840               | 30,500           | 335,340          |
| Conversion of Programme<br>Related Investment to<br>distributable reserves |          | 378,476               | -                | 378,476          |
| <b>Total expenditure</b>   | <b>5</b> | <b>694,886</b>        | <b>30,500</b>    | <b>725,386</b>   |
| <b>Net movements in funds</b>  |          | <b>(98,949)</b>       | <b>(30,000)</b>  | <b>(128,949)</b> |
| <b>Fund balances brought forward<br/>1 January 2017</b>                    |          | <b>1,670,795</b>      | <b>30,000</b>    | <b>1,700,795</b> |
| <b>Fund balance carried forward<br/>31 December 2017</b>                   |          | <b>1,571,846</b>      | <b>-</b>         | <b>1,571,846</b> |

► All transactions are derived from continuing activities.

## BALANCE SHEET AS AT 31 DECEMBER 2018

|   | Notes | 2018<br>£        | 2017<br>£        |
|---|-------|------------------|------------------|
| <b>Fixed assets</b>                                   |       |                  |                  |
| Tangible fixed assets                                 | 11    | -                | -                |
| Programme Related Investments                         | 12    | 3,643,399        | 712,462          |
| <b>Current assets</b>                                 |       |                  |                  |
| Debtors   | 13    | 1,213            | 383,066          |
| Cash at bank and in hand                              |       | 2,652,486        | 486,393          |
|   |       | 2,653,699        | 869,459          |
| <b>Creditors:</b> Amounts falling due within one year | 14    | (526,016)        | (10,075)         |
| <b>Net current assets</b>                             |       | 5,771,082        | 1,571,846        |
| <b>Net assets</b>                                     |       | <b>5,771,082</b> | <b>1,571,846</b> |
| <b>FUNDS</b>  |       |                  |                  |
| Restricted funds                                      | 15    | 520              | -                |
| Unrestricted general funds                            |       | 5,770,562        | 1,571,846        |
|   | 17    | <b>5,771,082</b> | <b>1,571,846</b> |

The financial statements were approved on 15/08/19 and signed on behalf of the Board by:

Simon Kanter - Chairman  
Trustee

Daniel Baum - Treasurer  
Trustee

## STATEMENT OF CASH FLOWS AS AT 31 DECEMBER 2018

|  | Notes     | 2018<br>£        | 2017<br>£      |
|--|-----------|------------------|----------------|
| <b>Cash flow from operating activities</b>           | <b>18</b> | 380,637          | (754,604)      |
| <b>Net cash flow from operating activities</b>       |           | 380,637          | (754,604)      |
| <b>Cash flow from investing activities</b>           |           |                  |                |
| Interest received                                    |           | 813              | 513            |
| Programme Related Investment dividends receivable    |           | 1,784,643        | 378,476        |
| <b>Net cash flow from investing activities</b>       |           | 1,785,456        | 378,989        |
| <b>Net increase in cash and cash equivalents</b>     |           | 2,166,093        | (375,615)      |
| <b>Cash and cash equivalents at 01 January 2018</b>  |           | 486,393          | 862,008        |
| <b>Cash and cash equivalents at 31 December 2018</b> |           | <b>2,652,486</b> | <b>486,393</b> |
| <b>Cash and cash equivalents consists of:</b>        |           |                  |                |
| Cash at bank and in hand                             |           | 2,652,486        | 486,393        |
| <b>Cash and cash equivalent at 31 December 2018</b>  |           | <b>2,652,486</b> | <b>486,393</b> |

# NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

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## 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 2018

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### 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 5.

### 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.



# NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

## 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.

## 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.

## 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.

| <b>2. DONATIONS</b>                              | 2018<br>£        | 2017<br>£      |
|--|------------------|----------------|
| <b>Restricted funds:</b>                         |                  |                |
| Donations  | 5,560            | 500            |
| <b>Unrestricted funds:</b>                       |                  |                |
| Donations  | 34,506           | 49,817         |
| Dinner income 2013                               | -                | -              |
| Dinner income 2016                               | 3,250            | 40,151         |
| Yorkshire Three Peaks Challenge                  | 10,000           | 121,684        |
| Prudential RideLondon-Surrey                     | 32,463           | 296            |
|  | <b>85,779</b>    | <b>212,448</b> |
| <b>3. INVESTMENT INCOME</b>                      | 2018<br>£        | 2017<br>£      |
| Interest   | 813              | 513            |
| Programme Related Investment dividend receivable | 1,784,643        | 378,476        |
|  | <b>1,785,456</b> | <b>378,989</b> |

## NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

|   |                  |                   |                    |                    |                    |
|---|------------------|-------------------|--------------------|--------------------|--------------------|
| <b>4. OTHER INCOME</b>  |                  |                   |                    | 2018<br>£          | 2017<br>£          |
| <b>Unrestricted funds:</b>  |                  |                   |                    |                    |                    |
| Rental income   |                  |                   |                    | 700                | -                  |
| Dinner sponsorship  |                  |                   |                    | -                  | 5,000              |
|   |                  |                   |                    | <b>700</b>         | <b>5,000</b>       |
| <b>5. ANALYSIS OF EXPENDITURE</b>   | Staff costs<br>£ | Direct costs<br>£ | Support costs<br>£ | Total<br>2018<br>£ | Total<br>2017<br>£ |
| Raising funds   | -                | 3,693             | -                  | 3,693              | 11,570             |
| Charitable activities   | 46,171           | 532,897           | 20,875             | 599,943            | 335,340            |
| Conversion of Programme   | -                | -                 | -                  | -                  | 378,746            |
| Related Investments   | -                | -                 | -                  | -                  | -                  |
| <b>Total expenditure</b>  | <b>46,171</b>    | <b>536,590</b>    | <b>20,875</b>      | <b>603,636</b>     | <b>725,386</b>     |
| Support costs have all been allocated against charitable activities.                |                  |                   |                    |                    |                    |
| <b>6. RAISING FUNDS</b>   |                  |                   |                    | 2018<br>£          | 2017<br>£          |
| Website marketing   |                  |                   |                    | 114                | 540                |
| Prudential Bike Ride  |                  |                   |                    | 2,700              | -                  |
| Yorkshire Three Peaks Challenge   |                  |                   |                    | -                  | 8,316              |
| Other costs   |                  |                   |                    | 879                | 2,714              |
|   |                  |                   |                    | <b>3,693</b>       | <b>273,430</b>     |
| <b>7. DIRECT CHARITABLE EXPENDITURE</b>   |                  |                   |                    | 2018<br>£          | 2017<br>£          |
| <b>Restricted expenditure:</b>  |                  |                   |                    |                    |                    |
| Great Ormond Street Clinical Trial Co-ordinator                                     |                  |                   |                    | -                  | 30,000             |
| KineDMD – Imperial College London   |                  |                   |                    | -                  | 500                |
| Solid – Non-viral delivery research   |                  |                   |                    | 5,040              | -                  |
| <b>Unrestricted expenditure:</b>  |                  |                   |                    |                    |                    |
| KineDMD – Imperial College London   |                  |                   |                    | -                  | 269,500            |
| Blood-based biomarker   |                  |                   |                    | -                  | (84,233)           |
| Treat-NMD Programme Co-ordinator  |                  |                   |                    | 15,445             | -                  |
| HHMI Fellowship   |                  |                   |                    | -                  | 31,280             |
| Muscular Dystrophy Campaign   |                  |                   |                    | -                  | 5,852              |
| Newcastle University Clinical Research Fellowship (administered by Action Duchenne) |                  |                   |                    | 7,143              | 7,143              |
| World Muscle Society  |                  |                   |                    | 500                | 1,500              |
| Little Steps  |                  |                   |                    | -                  | 860                |
| Gene therapy for Becker – UCL   |                  |                   |                    | 81,142             | -                  |
| CRISPR for Duplications – Hospital for Sick Children, Toronto                       |                  |                   |                    | 108,667            | -                  |
| Solid – Non-viral delivery research   |                  |                   |                    | 314,960            | -                  |
|   |                  |                   |                    | <b>532,897</b>     | <b>335,340</b>     |

## NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

| <b>8. SUPPORT COSTS</b>           | 2018<br>£ | 2017<br>£ |
|-----------------------------------|-----------|-----------|
| Legal costs                       | 573       | 383       |
| Bank charges                      | 735       | 636       |
| Postage, stationery and telephone | 238       | 2,589     |
| Administration                    | 2,975     | 4,944     |
| Rent and service costs            | 8,288     | 9,020     |
| Travel costs                      | 559       | 3,773     |
| Insurance                         | 2,197     | 2,078     |
| Depreciation                      | -         | 287       |
| Other                             | 846       | 2,456     |
| Governance                        | 4,464     | 4,200     |

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|  |               |               |
|--|---------------|---------------|
|  | <b>20,875</b> | <b>30,366</b> |
|--|---------------|---------------|

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| <b>9. GOVERNANCE COSTS</b>         | 2018<br>£ | 2017<br>£ |
|------------------------------------|-----------|-----------|
| Auditors' remuneration - for audit | 4,464     | 4,200     |

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|  |              |              |
|--|--------------|--------------|
|  | <b>4,464</b> | <b>4,200</b> |
|--|--------------|--------------|

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| <b>10. STAFF COSTS AND TRUSTEE REMUNERATION</b> | 2018<br>£ | 2017<br>£ |
|---|-----------|-----------|
| Wages and salaries                              | 44,821    | 41,802    |
| Social Security costs                           | 776       | 623       |
| Pension Costs                                   | 574       | 147       |

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|  |               |               |
|--|---------------|---------------|
|  | <b>46,171</b> | <b>42,572</b> |
|--|---------------|---------------|

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Two members of staff (head count) were employed during 2018 (2017: Two). No employees earned over £60,000 per annum.

No benefits are received by key management personnel (2017: £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 £1,595 (2017: £1,329) was paid for Trustee Indemnity Insurance.

## NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

### 11. TANGIBLE FIXED ASSETS

|                                      | Furniture and<br>equipment<br>£ |
|--------------------------------------|---------------------------------|
| <b>Cost:</b>                         |                                 |
| At 1 January 2018 & 31 December 2018 | 3,547                           |
| <b>Depreciation:</b>                 |                                 |
| At 1 January 2018 & 31 December 2018 | 3,547                           |
| <b>Net book value:</b>               |                                 |
| At 1 January 2018 & 31 December 2018 | -                               |

### 12. PROGRAMME RELATED INVESTMENTS

|  | 2018<br>£        | 2017<br>£      |
|--|------------------|----------------|
| Cost of investment at 1 January 2018                       | 712,462          | 1,090,938      |
| Conversion of investment to distributable reserves in DRIL | -                | (378,476)      |
| Gains on investment  | 2,930,937        | -              |
|  | <b>3,643,399</b> | <b>712,462</b> |

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 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 2018.

During the year DRIL declared a dividend to Duchenne Research Fund of £1,784,643 (2017: £378,476).

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

### 13. DEBTORS

|                     | 2018<br>£    | 2017<br>£      |
|---------------------|--------------|----------------|
| Gift Aid receivable | 1,213        | 4,590          |
| Other debtors       | -            | 378,476        |
|                     | <b>1,213</b> | <b>383,066</b> |

## NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

| <b>14. CREDITORS</b> Amounts falling due within one year | 2018<br>£      | 2017<br>£     |
|--|----------------|---------------|
| Accruals   |                |               |
| Other taxes and social security                          | 6,456          | 8,533         |
| Grants payable   | 2,108          | 1,542         |
|  | 517,452        | -             |
|  | <b>526,016</b> | <b>10,075</b> |

| <b>15. RESTRICTED FUNDS</b> | Balance at<br>1.01.18<br>£ | Income<br>£  | Expenditure<br>£ | Balance at<br>31.12.18<br>£ |
|-----------------------------|----------------------------|--------------|------------------|-----------------------------|
| 2018                        |                            |              |                  |                             |
| Research grants             | -                          | 5,560        | (5,040)          | 520                         |
|                             | <b>-</b>                   | <b>5,560</b> | <b>(5,040)</b>   | <b>520</b>                  |

|                 | Balance at<br>1.01.17<br>£ | Incoming<br>resources<br>£ | Resources<br>expended<br>£ | Balance at<br>31.12.17<br>£ |
|-----------------|----------------------------|----------------------------|----------------------------|-----------------------------|
| 2017            |                            |                            |                            |                             |
| Research grants | 30,000                     | 500                        | (30,500)                   | -                           |
|                 | <b>30,000</b>              | <b>500</b>                 | <b>(30,500)</b>            | <b>-</b>                    |

## 16. RELATED PARTY TRANSACTIONS

One trustee, Sarah Shelley, was a director of Duchenne Research (UK) Investment Ltd ("DRIL"). During the year, dividends totalling £1,784,643 were receivable (2017: £378,476).

| <b>17. ANALYSIS OF NET ASSETS BETWEEN FUNDS</b> | Restricted<br>2018<br>£ | Unrestricted<br>2018<br>£ | Total<br>2018<br>£ |
|---|-------------------------|---------------------------|--------------------|
| 2018  |                         |                           |                    |
| Fixed assets                                    | -                       | 3,643,399                 | 3,643,399          |
| Current assets                                  | 520                     | 2,653,179                 | 2,653,699          |
| Current liabilities                             | -                       | (526,016)                 | (526,016)          |
|   | <b>520</b>              | <b>5,770,562</b>          | <b>5,771,082</b>   |

|                     | Restricted<br>2017<br>£ | Unrestricted<br>2017<br>£ | Total<br>2017<br>£ |
|---------------------|-------------------------|---------------------------|--------------------|
| 2017                |                         |                           |                    |
| Fixed assets        | -                       | 712,462                   | 712,462            |
| Current assets      | -                       | 869,459                   | 869,459            |
| Current liabilities | -                       | (10,075)                  | (10,075)           |
|                     | <b>-</b>                | <b>1,571,846</b>          | <b>1,571,846</b>   |



## NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2018

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| <b>18. RECONCILIATION OF (EXPENDITURE) / INCOME<br/>TO CASH FLOW FROM OPERATING ACTIVITIES</b> | <b>2018<br/>£</b> | <b>2017<br/>£</b> |
|--|-------------------|-------------------|
| Net (expenditure) / income for 31 December 2018  | 1,268,299         | (128,949)         |
| Interest received  | (813)             | (513)             |
| Dividends received   | (1,784,643)       | -                 |
| Depreciation   | -                 | 287               |
| Decrease / (increase) in debtors   | 381,853           | (312,672)         |
| Increase / (decrease) in creditors   | 515,941           | (312,757)         |
| <b>Net cash flow from operating activities</b>   | <b>380,637</b>    | <b>(754,604)</b>  |