

Batten Disease Family Association

Annual Report and Financial Statements

for the Year Ended 31 August 2016

Charity Number : 1084908

Batten Disease Family Association
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REGISTERED CHARITY NUMBER 1084908

**REPORT OF THE TRUSTEES AND UNAUDITED FINANCIAL STATEMENT FOR THE
YEAR ENDED 31 AUGUST 2015 FOR BATTEN DISEASE FAMILY ASSOCIATION
(BDFA)**

The Trustees present their report with the financial statements of the charity for the year ended 31 August 2016.

REFERENCE AND ADMINISTRATIVE DETAILS

Registered Charity Number

1084908

Principal Address

The Old Library
4 Boundary Road
Farnborough
Hampshire
GU14 6SF

Trustees

Chair	Michael O'Connor	
Co-Chair	Pauline Docherty	
Secretary	Sarah Kenrick	
Treasurer	Ben Jones	
	Barbra Higgins	
	Shaun Andrews	
	Ellen Bletsoe	
	Catherine Sermon	
	James Jeynes	Co-opted 2016
	Roger Cole	Co-opted 2016
	Neil Dunford	Co-opted 2016
	Dave Mitchell	Co-opted 2016

Independent Examiner

Hilary Parmenter
Chartered Accountant
1 The Willows
North Warnborough
Hampshire RG29 1DR

Bankers

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

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L30 4GB

REFERENCE AND ADMINISTRATIVE DETAILS

Research Advisors

Professor Sara Mole
MRC Laboratory for Molecular Cell
Biology
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Gower Street
London
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Dr Brenda P Williams
Senior Lecturer
Programme Leader, MSc Neuroscience
Distance Learning
KATTP Summer School Lead
Departmental Teaching Lead
IoPPN Assessment and Feedback Lead
Institute of Psychiatry, Psychology &
Neuroscience
Department of Basic and Clinical
Neuroscience
Maurice Wohl Clinical Neuroscience Institute
5 Cutcombe Road
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Biomedical Sciences
Department of Comparative Biomedical
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Los Angeles Biomedical Research Institute
at Harbor-UCLA Medical Center
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Education Adviser

Mrs Barbara Cole, Education Advocate

Medical Adviser

1. Professor Paul Gissen,
Consultant in Paediatric Metabolic Disease,
Great Ormond Street Children's Hospital
London
and
Wellcome Trust Senior Research Fellow in
Clinical Sciences at UCL Institute of Child
Health.

2. Dr Barbara Csanyi
Paediatric Neurologist
Great Ormond Street Children's Hospital
London

3. Dr Christine Caren
GP (retired)

STRUCTURE, GOVERNANCE AND MANAGEMENT

Governing Document

The Batten Disease Family Association was formed in November 1998 and became a registered unincorporated Charity on 7th February 2001 constituted as an Association. Charity number 1084908.

Recruitment and Appointment of New Trustees

The BDFA values a diverse Board and recruits trustees based on their experience and on the skills and expertise they can bring to the Charity. The Board maintains a balance between those who have direct experience of Batten disease as family members and those with other skills relevant to the development of the Charity mission and aims. Advertisements for new trustees are made in the Association's newsletter, with volunteer bureaux, on the BDFA website and social media platforms.

Candidates are then given information on the roles, interviewed by the current Board and invited to attend a Trustees' meeting. Appointment is then conditional on the approval of Trustees and members at the AGM and is subject to a DBS disclosure and Trustee's Declaration of Eligibility. Trustees have fully documented information packs and job descriptions to support their recruitment and induction to the Board.

Organisational Structure

The Strategy and oversight of the affairs of the Batten Disease Family Association are directed by the Trustees. The day to day management of the Batten Disease Family Association has been carried out by the employed Chief Executive, Andrea West, and supported by the following team in 2015/2016:

1. Harriet Lunneman (Full-time) Family Support Officer
2. Heather Band (32 hours per week) Scientific Officer
3. Lucy Roose (25 hours per week) Office Manager
4. Gaynor Heeson (20 hours per week) Fundraising Administrator
5. Kate Shefford (20 hours per week) Fundraising Administrator
6. Laura Codd (7.5 hours per week) BATCure Administrator

All HR and Health and Safety procedures for the BDFA are done under contract with Peninsula Business Services.

The BDFA has a team of volunteers who assist with office administration and fundraising activities. All volunteers have a defined induction and undergo DBS checks where appropriate. The Batten Disease Family Association also benefits from the services of volunteer educational, medical and research advisors who liaise with the employed Officers and the relevant Trustees as necessary.

The Board of Trustees meets 6 times per year with the Chief Executive to review work and formulate strategy.

Risk Management

The BDFA Board of Trustees places a high priority on risk management. Risks identified are categorised as follows:

- Governance and compliance
- Operational
- Financial
- External and compliance

These risks are reviewed as part of an annual process and as a result of this process the Board is satisfied that the residual risks are minimal.

OBJECTIVES AND ACTIVITIES

Who we are

The Batten Disease Family Association is the only UK charity supporting families, raising awareness and funding research into the devastating set of neurodegenerative diseases Neuronal Ceroid Lipofuscinoses (NCL), commonly known as Batten disease. The BDFA was founded in 1998 by a group of parents who were determined that no family should face the journey with this diagnosis alone. We work with the scientific, medical, social care and educational professionals in the UK and worldwide to drive forward towards therapies for all forms of the disease and to ensure that families living with the diagnosis today have access to the best services available.

About the NCLs (Batten disease)

The NCLs (Batten disease) are several different genetic, life-limiting neurodegenerative diseases that share similar features and occur in children and adults worldwide.

The NCLs (Batten disease) are estimated to affect 1:30,000 births with there being approximately 200 affected children and young adults in the UK. They are born apparently healthy, before going on to develop epilepsy, lose their sight and speech, their cognitive and motor abilities; and then dying between the ages of 5 and 30 (depending on the specific diagnosis). There continues to be much research into all forms of the NCLs, but no cure at present.

An NCL diagnosis profoundly changes the child's and their family's life forever. The journey with these diseases is an ever-changing picture of needs. Unlike other diseases where there is huge awareness of the support, therapies and guidance resources needed for the child and family, the families of children and young people with Batten disease are left isolated as most professionals do not understand the course of the disease and the child or family's needs. It is therefore essential that the families and professionals have a place to turn to for that support and guidance.

The BDFA produces valuable resources, materials and training for families and professionals on all aspects of the NCLs and the holistic care needed for affected children and young adults.

What we do

Support: Our aim is that no family faces the devastating journey with Batten disease alone. Through our family support and advocacy service we aim to improve the quality of life for families, affected children and adults and also the teams of professionals around them.

Advocacy and awareness: We aim to raise awareness of this set of rare diseases. We also aim to influence policy and service provision for all forms of the NCLs to enable families and professionals to access the best resources and to give the best quality of life to affected children and young adults.

Research funding: We aim to provide funding for vital pre-clinical and clinical research in universities and research institutions both in the UK and worldwide. Research is vital to both determine the basic mechanisms of the disease and to develop effective therapies and ultimately a cure.

Public benefit: In making decisions regarding the charities purposes and what the charity has done throughout the year to carry out those purposes the trustees have had due regard to the commission's public benefit guidance when exercising any powers or duties to which the guidance is relevant.

Letter from BDFA Chair of Trustees, Mike O'Connor

I would like to share with you the work of the BDFA over the last year with families affected by a diagnosis of Batten disease and the teams of professionals who work with them.

In 2014-2015 we were able to recruit the first UK Batten disease Clinical Nurse Specialist, Katie Hanson continued to consolidate this role working with many families both face to face and remotely providing clinical support and advice whilst developing a strong professional network across the U. After she left the role we then recruited Laura Lee to the post at Great Ormond Street Hospital to continue to provide this invaluable service to families and professionals. Laura comes from working at the Somers Clinical Research Facility at GOS and met families there taking part in the BioMarin CLN2 Enzyme Replacement Trial. We added another new member to the BDFA team this year when Laura Codd joined us as the BATCure administrator. The second meeting of the BATCure consortium took place in Riga in 2016 and more about the project can be found in the BDFA research funding section of this report.

This past year also saw the BDFA expand the education advocacy service we are able to offer to families to ensure that children and young people with an NCL diagnosis have the best education experience for them and their families. We are working with SENSE to provide further staff training in education advocacy to enable us to meet a growing needs as statutory services are cut and families struggle to get the support they need. We delivered 3 workshops and training days to education professionals across the country and plan more for the future.

Over the last year we have seen further progress with the BMN190 Clinical Trial by the US pharmaceutical company, BioMarin, for CLN2 (Late Infantile Batten disease). Data was presented at the World LSD Conference in San Diego showing that 87% of children on the trial experienced a slowdown in the progression of the disease. We continue to support all of those families enrolled at the sites in London, Hamburg and Rome for those families who continue on the extension study, and compassionate use. We also have worked closely with UK regulators to ensure that a safe, effective treatment is available to as many children as quickly as possible.

The BDFA's strong commitment to research into all forms of the NCLs has continued this year, with further funding of our key Gene Therapy to Treat Visual Failure project at the Institute of Ophthalmology, University College London. In January this year Sophia Kleine Holthaus was awarded her PhD. This was a very proud moment for the BDFA as Sophia is the first student to obtain a doctorate on a BDFA commissioned project. BDFA involvement in research has increased, both in the UK and worldwide in the fields of drug discovery, International NCL disease registry and research into the mechanisms of many forms of the NCLs. We continue to drive forward a research agenda to bring the hope of a cure for all forms of this disease a step closer.

I would like to thank everyone and please keep spreading the word, baking your cakes, running or cycling long distances and achieving amazing things in the hope that one day none of us will have to live with the horrors of Batten disease. We still have a way to go but we are making progress together and we can be very proud of what we have done and what we continue to do.

ACHIEVEMENT AND PERFORMANCE

BDFA Support and Advocacy Programme

The BDFA delivers a support and advocacy programme centred on the reported needs of UK families affected by a Batten disease diagnosis. In 2016 this support has expanded to cover contact from and support given to families and professionals from over 30 countries worldwide. Given the rare nature of the NCLs it is also important that we are able to provide a programme of support, training and networking to the teams of professionals around families. Only by driving this holistic programme of care are we able to meet the needs that families report to us to enable them and their children and young people to live the best life they can.

Some highlights of the past year are:

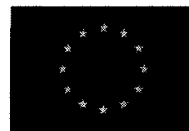
1. Consolidation of the support programme by the BDFA support and advocacy worker, Harriet Lunneman - a qualified social worker with extensive experience of working with children with disabilities.
2. Funding and recruitment of a Batten disease Clinical Nurse Specialist – Laura Lee - at Great Ormond Street Children's Hospital in London.
3. The Support and Advocacy role continues to provide an advocacy and signposting service through the BDFA Freephone helpline and in face to face meetings with both families and professionals supporting over 100 families throughout 2016.
4. Collaborative work with the US Pharmaceutical firm BioMarin to deliver a support service to those families enrolled on the BMN190 (and follow-up trials) of enzyme replacement therapy for CLN2 disease in the trial sites in London, Rome and Hamburg.
5. In 2015/16 the BDFA small grant scheme provided 15 families with much needed financial support for items such as driving lessons, passports, play equipment, educational equipment and washing machines.
6. Continuing to provide much needed education advocacy support through our education advisor to ensure that children and young people with an NCL diagnosis receive the best education experience, delivering 3 training workshops to education professionals.
7. Delivery of a Family Networking Conference in Coventry, which saw an increased attendance from families and the professionals working with them with 150 individuals. They attended an extensive programme of workshops and presentations covering all aspect of care for children, research updates and a comprehensive programme of childcare and activities for both affected children and siblings.
8. Updates and new material added to the Family Resources folder based on family feedback. This is free to all family members of the BDFA and provides information on the disease, symptoms, progression, health and social care, education and resources available.
9. New information leaflets on feeding and gastrointestinal symptoms was produced for families.
10. We continue to publish a bi-annual member and supporter newsletter. We also send weekly email updates on our work, impact and valuable resources to members which now reaches over 1500 subscribers.
11. We maintain a strong presence on a number of social media platforms to raise awareness, connect families and fund our work.
12. We promote links between UK professionals with clinical, research and educational specialist expertise in Batten disease and those who can give professional guidance to affected families and the teams around them.

13. We work closely with other charities and stakeholders such as The Amber Trust, Dreams Come True, Make a Wish and Roald Dahl's Marvellous Children's Charity to provide much needed therapies and experiences to affected children and young adults.

BDFa Research Programme

Finding successful treatments and potential therapies for all forms of the NCLs is a central aim of the BDFa and we support research for all stages of this process. The BDFa place equal importance on projects which look holistically at all aspects of Batten disease and the lives of families living with it.

The BDFa is constantly active in promoting awareness of Batten disease amongst clinicians, researchers and other related professionals and works with many difference organisations to drive forward rare disease research. The BDFa also has a comprehensive programme of conference and meeting sponsorship.



1. BATCure (2015-2018)

This project has received funding from the European Union's Horizon2020 research and innovation programme under grant agreement No 666918

The goal of the 3- year project is to advance the development of new therapeutic options for patients and their families living with CLN3, CLN6 or CLN7 Batten disease.

The consortium is made up of groups from seven European countries, co-ordinated by Prof. Sara Mole at UCL, including ten leading scientific research groups, three companies and the BDFa.

The BDFa is leading part of the project ensuring that the voice of patients and affected families is heard:

- Develop a methodology to enable patients, patient groups & stakeholders to participate in the project.
- Prepare for future Clinical Trials
- Communication strategy with Public & professional engagement
- Exploitation and Dissemination strategy

The funding has provided for 1 day per week of BDFa SO time and the recruitment of an administrator. Laura Codd joined the BDFa in April 2016.

2. JNCL and Education Project, Erasmus+, European Union (EU)

The BDFA continues to be the patient advocacy lead for this project in collaboration with 6 other European countries. The BDFA delivered the survey of the educational experiences of families with a child or young person affected by CLN3, in the UK. For the final stage of the project we are bringing our considerable expertise to the design of educational tools and a publication on working in education with young people with CLN3 (Juvenile Batten Disease)

3. MIND Music Therapy Project

The BDFA is a key stakeholder in a music therapy project with Prof. Adam Ockelford, University of Roehampton and Chiltern Music Therapy, investigating the role of music in the lives of children and young people with Batten disease.

4. CLN2 Burden of Disease Project

In 2016, the BDFA delivered the CLN2 Burden of Disease Project with BioMarin, investigating the emotional, physical and financial burdens of caring for a child or children with CLN2 (Late Infantile Batten disease). This work has been accepted as an oral presentation by Andrea West, BDFA CEO at the 15th International NCL conference, Boston 2016.

5. CLN2 Care Management Guidelines

In 2016 the BDFA contributed with other leading NCL clinical and social care professionals from across the world to the development of the first consensus document on care guidelines for children with CLN2 (Late Infantile Batten disease).

6. International NCL Disease Registry

Dr. Angela Schultz, MD, Lead Coordinator DEM-CHILD NCL patient database consortium
University Medical Center Hamburg-Eppendorf
BDFA £11 500 contribution to BDSRA awarded research grant of \$55 000.
Dr Suzanne Lezius, bio-mathematician Mar 2016, 1-year

This project is being undertaken by the DEM-CHILD NCL patient database consortium, which was initially founded by members of the European Commission. The aim is to collect the world's largest, clinically and genetically best characterized, set of NCL patient data. These data collections will be an indispensable tool for the evaluation and validation of new therapies and the validation and improvement of palliative care of NCL patients.

7. Gene therapy to treat vision loss in NCL (Batten disease), CLN3, CLN6 and CLN7 and CLN2.

Dr Sara Mole, MRC Laboratory for Molecular Cell Biology, UCL
Prof Robin Ali, Dr Alexander Smith, Institute of Ophthalmology, UCL.
£72 000 2016-2019 (with Wellcome Trust, £112 000)

Vision loss is a key symptom of Batten disease and so the quality of life of those affected would improve if at least some vision could be preserved. The aim of this project is to investigate the feasibility of gene therapy to treat visual failure in Batten disease.

Sophia kleine Holthaus (BDFA 3-year PhD studentship Jan 2012-Dec 2014) was formally awarded her doctorate in January 2016. Sophia's excellent work for her PhD project showed that whilst loss of photoreceptor cells in the eye occurred early on in NCL disease, other cell populations were affected and this would need to be addressed for a successful therapeutic outcome.

The BDFA funded a further 1-year project in January 2015. This enabled the work to continue without interruption with the added benefit that the knowledge and skills of an exceptional researcher would be retained within the field. In 2015 Sophia's work has demonstrated a positive effect in the treatment of the cells identified as crucial in treating vision loss in her mouse model of Batten disease. Further work will be needed to confirm and build upon this research and translate it to other forms of NCL.

This is now possible due to BATCure with a further 3-year award enabling Dr. Holthaus to complete her CLN6 studies and to extend her work to CLN3 and CLN7.

The BDFA has partnered with the Wellcome Trust to fund a second project for 3 years, 2016-9 (including £20 000 from Beefy's foundation) to cover CLN2, and dependent on how this proceeds to include other rarer NCLs. Dr. Mikel Aristorena was appointed in July 2016.

8. Drug discovery in a CLN2 zebrafish model

Dr Claire Russell, Gini Brickell, MSc Royal Veterinary College
Jan-October 2015 ,£19692.75, March-May 2016, £4982.00
(Freeman family, CLN2 restricted funds and BDFA research funds)

In 2013 the BDFA made an award to Dr Russell of £13,500 with funds provided by the Freeman family in memory of their daughter Katie, to provide the capacity to perform high-throughput in vitro drug discovery for LINCL (CLN2 disease) with the purchase of a Daniovision automated tracking system.

In 2015 a Research Associate (RA) was appointed to perform the drug screen. From this work a lead compound was identified (RVC1), as showing promise in a zebrafish model of CLN2 to improve key features of the disease. Dr Russell was further funded to undertake metabolomics studies (with AcureOmics, BATcure consortium member). Based on these findings the BDFA is currently supporting Dr. Russell to apply for further funding to complete mouse studies for RVC1.

9. Uncovering fundamental difference in the cell biology and biochemistry of CLN5 disease

Dr Emyr Lloyd-Evans, Cardiff University
£25 000 (funded on behalf of Battle Batten with matched funding from Cardiff university)
Oct 2016-2018

The aim of this 3-year co-funded PhD studentship is to identify key differences in cells made from CLN5 patients compared to healthy unaffected cells. In the first year key changes have already been found and further work is required to see if these can be exploited as potential therapeutic targets.

10. Development of a drug screen for CLN5 Batten disease

Professor Paul Gissen, Dr. Dan Little, Prof. Sara Mole, and Dr Robin Ketteler, UCL
£20 000 BDFA funded on behalf of Battle Batten
July 2016 1-year with 1-year extension

The aim of this project is to use patient's skin cells, which have the mutation or "mistake" in the CLN5 gene to create a type of cell (iPS cells) that can then be turned into nerve cells, the cells that die in CLN5 disease. These can then be used to develop a drug screen to find treatments that could make the cells healthier.

11. Assessing the efficacy of a Gene Therapy approach upon the neuropathology in CLN5 deficient sheep.

Prof. Jon Cooper, UCLA, Ana Assis, Kings College London (in collaboration with Prof. David Palmer, Dr. Nadia Mitchell, Lincoln, NZ)
£50 000 March 2016 1- year research assistant, administered by Sparks, The Children's Medical research charity (no 1003825)

The aim of this project is to provide histological analysis from CLN5 sheep treated at Lincoln University to determine if the gene therapy approach has been successful.

12. Cell based systems for drug discovery in JNCL

Professor Jon Cooper, Dr. Tytus Murphy, (Dr. Brenda Williams, Dr. Greg Anderson, until March 2014), Kings College London
£ 38622.00, 1-Year grant March 2013- Feb 2014, Extended to July 2016

In the 6-month extension period Dr. Murphy successfully demonstrated the presence of a human neuronal cell line containing the most common CLN3. As Prof. Cooper has moved to a new post at UCLA, the cell line has been transferred to Dr Tristan McKay, Manchester Metropolitan University (BATCure consortium member) for further studies.

OUR FUTURE PLANS

The work continues for the BDFA to enable us to further support families living with this diagnosis and legacy whilst working towards our vision of a world without Batten disease. The BDFA Board continue to prioritise our strategic planning to ensure a robust service that meets the needs of families and drives the research agenda for the future.

Goal 1: To ensure that everyone living with a diagnosis of Batten disease has access to high quality services and support to enable them to live life to the full.

To achieve this, we will:

- develop a national Batten disease service from screening through to diagnosis and potential therapies.
- advocate with national Government on the level and quality of service received by families living with a diagnosis of Batten disease.
- work with statutory and third sector providers to ensure that families receive a person-centred, needs led, high quality level of service across health, education and social care.

By 2018 we will:

- Having implemented and consolidated the first paediatric Batten disease Clinical Nurse Specialist in the UK.
- review and drive national advocacy and the position of Batten disease on the UK Health and Social care political agenda to initiate change and service development
- continue to have in place a Batten disease Support and Advocacy service across the UK to meet the needs of the whole of the family's journey with this disease from diagnosis through palliative care and bereavement.
- develop and implement an education advocacy service in the face of increasing need alongside the development of a Batten disease Education Professionals network
- support and continue to develop networking opportunities for families and the professionals who work with them based on the needs identified by these stakeholders

Support goals 2016-2018

- To provide a high quality support, signposting and referral service for families through one to one Support and Advocacy service
- To produce a Batten resource pack for each affected family
- To expand the Batten bereavement service offered to families throughout the UK
- To deliver 3 workshops per year of training and support for education, social care and health professionals working with affected families
- To develop and deliver a family needs assessment to better understand the needs of families living with an NCL diagnosis.
- To initiate weekly contact with new and existing allied organisations to share and develop a high quality resource library for families and professionals
- To consolidate and provide an induction for first Batten CNS post and develop programme of integrated working with BDFA
- To recruit a further 2 support and advocacy workers by 2018, one in the midlands and one in Scotland/North of England
- To hold an annual family conference that provides networking opportunities, research updates, education and clinical support.
- To hold a review of family networking scheme

- Annual update of all BDFA resources including online, signposting and resource pack.
- To develop diagnostic leaflet in collaboration with GOSH diagnostic team.
- With clinicians, develop clinical care pathways for all those with an NCL diagnosis.

Goal 2: To change lives by funding research into potential therapies and ultimately a cure.

To achieve this, we will:

- directly support the scientific community to drive innovative research through individual grants and collaborative funding
- develop and maintain strong research networks both in the UK and worldwide, attracting new and cutting-edge investigators;
- engage with regulatory authorities in the UK (MHRA) and Europe (EMA) to promote faster translation to clinical trials
- engage with pharmaceutical companies to drive the clinical research agenda for all the NCLs

By 2018 we will:

- spend £300k on research including the development of a collaborative funding relationship with Sparks.
- inspire existing and new funders and donors to invest in this most critical need.
- engage with Universities to source matched funding for research projects
- work with our partners in the scientific, clinical and pharmaceutical communities to campaign for the introduction of clinical trials and so to enable affected children and young people to access potential studies.

Research goals 2015-2018

- Collaboration with BioMarin to deliver a safe, effective licensed therapy for CLN2 (Late Infantile Batten disease)
- Represent BDFA and BDIA at key CLN2 expert meetings with BioMarin
- Co-ordinate and deliver CLN2 Burden of disease project in the UK with ICON and BioMarin
- Plan and deliver BDIA London meeting
- Play a key role in and host Erasmus+ JNCL European Education Project meeting in London
- £142k funding of Gene Therapy to treat visual failure at UCL
- Deliver £50k of research funding through Spark's partnership
- Deliver £70K of research funding for CLN5 research with Battle Batten campaign (Dawkins family)
- Deliver £25K of research funding for CLN2 with Freeman Family
- Review ongoing research projects for recommendation or decline of further funding
- Engagement with and attendance at meetings with relevant Professional bodies, and to advocate the patient view at scientific and clinical expert meetings in the UK and worldwide
- Contribute Patient Advocacy to the Batcure (UCL) Horizon2020 application for European Union Funding

- Direct funding for the DEM-CHILD NCL European registry project
- Resource and support UK patient recruitment to DEM-CHILD NCL European registry project
- engage with pharmaceutical start-ups who have expressed an interest in NCL therapeutics developments and continue to identify other potential commercial interest

Goal 3: To raise awareness of Batten disease on an UK national and international platform

To achieve this, we will:

- aid and inspire those both within and outside of the Batten community to improve the lives of those affected.

By 2018 we will:

- develop and implement a high profile multimedia strategy across the digital space and in local and national press.
- advanced NCL awareness in the legislative and governmental sphere.

Awareness goals 2016-2018

- Daily updates made to BDFA website to include enhanced news, information, support, fundraising and research resources
- Daily updates to social media pages to significantly increase social media profile
- Weekly mailchimp email to all subscribers
- Deliver staff training on website maintenance
- Monthly review of website and social media analytics
- Produce twice yearly newsletter and Batten bulletin
- Yearly update of BDFA media kit
- Secure quarterly opportunities to engage with local and national media around family, organizational and industry stories
- Engage with key NHS figures to raise the profile of the NCLs to advocate for better services and implementation of potential therapies
- Develop database of Members of Parliament with affected families in their constituency
- Attend meetings of LSD Collaborative, Genetic Alliance UK, Neurological Alliance and Rare Disease UK
- Implement programme of awareness raising for Rare Disease Day and Batten disease awareness Day

Organisational capacity.

Underpinning the strategic aims and goals of the BDFA is a programme to ensure organisational integrity, income development and service delivery effectiveness.

To achieve this, we will:

- Ensure that the BDFA's operations are compliant with all applicable laws and policies.
- Deliver a successful and dynamic fundraising strategy that will enable the BDFA to deliver its vision.
- Develop funds through diversified income streams to support BDFA programmes and

activities.

- Monitor and evaluate all aspects of BDFA service and activity to ensure best practice, and optimize use of resources.

By 2018 we will:

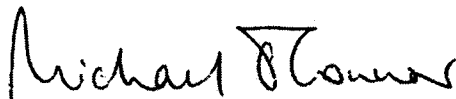
- Consolidation of offices and procedures to provide a secure base for strategic organizational growth
- Expanded and consolidated the BDFA staff team to meet growing demand for support and fundraising.
- Develop and review monitoring and evaluation systems for all aspects of BDFA service delivery.

Organisational goals 2016-2018

- Conduct annual accounts audit and Charity Commission filings in professional and timely manner.
- Implement HR requirements for staff and office location to support staff recruitment and development.
- Renew contracts on renewal to assure best pricing
- Deliver BDFA Fundraising Strategy
- Develop and implement a Trustee board recruitment and review process to ensure strong governance for the BDFA
- Recruitment of new medical, education and research advisors to the BDFA team to support and strengthen the professional network available to service users
- Qualitative and quantitative metrics compiled monthly on support service delivery including service user feedback.
- Review and application for potential accreditations for BDFA services

Approved by order of the board of trustees on23rd October 2016..... and signed on its behalf by:

Michael O'Connor (BDFA Chair of Trustees)



**Independent Examiner's Report to the Trustees of the
Batten Disease Family Association
Charity Number 1084908**

I report on the accounts for the year ended 31 August 2016, which are set out on pages 18 to 27.

Respective Responsibilities of the Trustees and Independent Examiner

The Trustees are responsible for the preparation of the accounts. The Trustees consider that an audit is not required for this year under section 144(2) of the Charities Act 2011 (the Charities Act) and that an independent examination is needed.

It is my responsibility to:

- examine the accounts under section 145 of the Charities Act;
- follow the procedures laid down in the general Directions given by the Charity Commissioners under section 145(5)(b) of the Charities Act; and
- to state whether particular matters have come to my attention.

Basis of Independent Examiner's Statement

My examination was carried out in accordance with the General Directions given by the Charity Commission.

An examination includes a review of the accounting records kept by the charity and a comparison of the accounts presented with those records. It also includes consideration of any unusual items or disclosures in the accounts, and seeking explanations from the trustees concerning any such matters. The procedures undertaken do not provide all the evidence that would be required in an audit and consequently no opinion is given as to whether the accounts present a 'true and fair' view and the report is limited to those matters set out in the statement below.

Independent Examiner's Statement

In connection with my examination, no matters have come to my attention:

(1) which give me reasonable cause to believe that in any material respect the requirements

- to keep accounting records in accordance with section 130 of the Charities Act; or
- to prepare accounts which accord with these accounting records and comply with the accounting requirements of the Charities Act

have not been met; or

(2) to which, in my opinion, attention should be drawn in order to enable a proper understanding of the accounts to be reached.



Hilary Parmenter
Chartered Accountant

8th November 2016

1 The Willows
North Warnborough
Hampshire
RG29 1DR

Batten Disease Family Association
Statement of Financial Activities for the Year Ended 31 August 2016

	Unrestricted Funds	Restricted Funds	Total Funds 2016	Total Funds 2015
Note	£	£	£	£
Incoming resources				
Incoming resources from generated funds				
Voluntary income	2	126,186	88,353	214,539
Activities for generating funds	3	113,775	-	113,775
Investment income	4	192	-	192
Total incoming resources		<u>240,153</u>	<u>88,353</u>	<u>328,506</u>
Resources expended				
Fundraising trading	5,6	43,169	-	43,169
Charitable activities	6	279,495	120,267	399,762
Governance costs	9, 6	8,868	-	8,868
Total resources expended		<u>331,532</u>	<u>120,267</u>	<u>451,799</u>
Net outgoing resources before transfers		(91,379)	(31,914)	(123,293)
Transfers				
Gross transfers between funds		<u>5,115</u>	<u>(5,115)</u>	<u>-</u>
Net movements in funds		(86,264)	(37,029)	(123,293)
Reconciliation of funds				
Total funds brought forward		<u>129,004</u>	<u>153,260</u>	<u>282,264</u>
Total funds carried forward		<u>42,740</u>	<u>116,231</u>	<u>158,971</u>

The notes on pages 20 to 27 form an integral part of these financial statements

**Batten Disease Family Association
Balance Sheet as at 31 August 2016**

		2016		2015	
Note	£	£	£	£	£
Current assets					
Debtors	12	6,511		6,438	
Cash at bank and in hand		<u>167,503</u>		<u>301,700</u>	
			174,014		308,138
Creditors: Amounts falling due within one year	13		(15,043)		(25,875)
Net current assets			<u>158,971</u>		<u>282,263</u>
Net assets	17		<u>158,971</u>		<u>282,263</u>
The funds of the charity:					
Restricted funds in surplus			116,436		163,884
Restricted funds in deficit					
Geri Lindup Effies fund			-		(2,164)
Biomarin			(205)		(6,514)
<i>In memory of Katie Freeman</i>			<u>-</u>		<u>(1,946)</u>
Total restricted funds			116,231		153,260
Unrestricted funds					
Unrestricted income funds			<u>42,740</u>		<u>129,003</u>
Total charity funds	16		<u>158,971</u>		<u>282,263</u>

The financial statements have been prepared in accordance with the Financial Reporting Standard for Smaller Entities (effective January 2015).

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

1 Accounting policies

Basis of preparation

The accounts (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 for Smaller Entities published on 16 July 2014, the Financial Reporting Standard for Smaller Entities (FRSSE), and the Charities Act 2011 and UK Generally Accepted Practice as it applies from 1 January 2015.

Fund accounting policy

Unrestricted income funds are general funds that are available for use at the trustee's discretion in furtherance of the objectives of the charity.

Restricted funds are those donated for use in a particular area or for specific purposes, the use of which is restricted to that area or purpose.

Further details of each fund are disclosed in note 17.

Incoming resources

Voluntary income including donations and legacies is recognised where there is entitlement, certainty of receipt and the amount can be measured with sufficient reliability.

Incoming resources from tax reclaims are included in the statement of financial activities at the same time as the gift to which they relate.

Shop income is recognised as earned (that is, as the related goods or services are provided).

Investment income is recognised on a receivable basis.

Batten Disease Family Association

Notes to the Financial Statements for the Year Ended 31 August 2016

..... *continued*

Resources expended

Liabilities are recognised as soon as there is a legal or constructive obligation committing the charity to the expenditure. All expenditure is accounted for on an accruals basis and has been classified under headings that aggregate all costs related to the category.

Costs of generating funds comprise the costs associated with attracting voluntary income and the costs of trading for fundraising purposes.

Charitable expenditure comprises those costs incurred by the charity in the delivery of its activities and services for its beneficiaries. It includes both costs that can be allocated directly to such activities and those costs of an indirect nature necessary to support them.

Grants payable are payments made to third parties in the furtherance of the charitable objectives. Where the charity gives a grant with conditions for its payment being a specific level of service or output to be provided, such grants are only recognised in the SoFA once the recipient of the grant has provided the specific service or output.

Grants payable without performance conditions are only recognised in the accounts when a commitment has been made and there are no conditions to be met relating to the grant which remain in the control of the charity.

Provisions for grants are made when the intention to make a grant has been communicated to the recipient but there is uncertainty about either the timing of the grant or the amount of grant payable.

Governance costs

Governance costs include costs of the preparation and examination of the statutory accounts, the costs of trustee meetings and the cost of any legal advice to the trustee on governance or constitutional matters.

Support costs

Support costs include central functions and have been allocated to charitable activities or fundraising expenses on a basis consistent with the use of resources, for example staff costs by the time spent and other costs by their usage.

Research and development expenditure

Research and development expenditure is written off as incurred.

Operating leases

Rentals payable under operating leases are charged in the statement of financial activities on a straight line basis over the lease term.

Pensions

The charity operates a defined contribution pension scheme. Contributions are charged in the statement of financial activities as they become payable in accordance with the rules of the scheme.

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

2 Voluntary income

	Unrestricted Funds £	Restricted Funds £	Total Funds 2016 £	Total Funds 2015 £
Donations				
Regular monthly donations	4,999	-	4,999	4,894
Donations gift aid	6,064	-	6,064	31,094
Donations non gift aid	3,021	3,300	6,321	2,557
Membership fees	-	-	-	
Charitable giving	35,653	85,053	120,706	188,143
Gift aid reclaimed	22,343	-	22,343	16,644
Donations - organisations and schools	17,879	-	17,879	17,595
Foundations and trusts not restricted	9,864	-	9,864	16,535
Workshop income	1,261	-	1,261	
Conference income	22,714	-	22,714	
	123,798	88,353	212,151	277,986
In memoriam donations				
In memorium	2,388	-	2,388	3,270
	126,186	88,353	214,539	281,256

3 Activities for generating funds

	Unrestricted Funds £	Restricted Funds £	Total Funds 2016 £	Total Funds 2015 £
Fundraising				
Supporter fundraising	61,428	-	61,428	20,974
Collection boxes	135	-	135	448
Fundraising activities	51,612	-	51,612	402
	113,175	-	113,175	21,824
Merchandise income				
Merchandise income	600	-	600	1,281
	113,775	-	113,775	23,105

4 Investment income

	Unrestricted Funds £	Restricted Funds £	Total Funds 2016 £	Total Funds 2015 £
Interest income	192	-	192	346

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

5 Fundraising expenses

	Unrestricted Funds £	Restricted Funds £	Total Funds 2016 £	Total Funds 2015 £
Fundraising				
Cost of goods sold	15,276	-	15,276	16,879
Employment costs	27,516	-	27,516	19,908
	42,792	-	42,792	36,787
Merchandise income				
Cost of goods sold	377	-	377	3,276
	43,169	-	43,169	40,063

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

6 Total resources expended

	Grants	Fundraising	Mercha ndise income	Charitable activity	Governance	Total
	£	£	£	£	£	£
Grants payable - institutions	107,335	-	-	-	-	64,335
Grants payable - individuals	4,779	-	-	-	-	4,779
Fundraising costs	-	-	-	29,571	-	29,571
Restricted costs	-	-	-	61,784	-	61,784
Fundraising expenses	-	15,276	-	(2,362)	-	12,914
Merchandise for resale	-	-	377	-	-	377
Education costs	-	-	-	6,617	-	6,617
Eurodis Expenditure	-	-	-	1,944	-	1,944
Employment costs	-	27,516	-	134,933	-	162,449
Rent	-	-	-	8,267	-	8,267
Insurance	-	-	-	731	-	731
Premises expenses	-	-	-	4,447	-	4,447
Telephone and fax	-	-	-	199	-	199
Office equipment	-	-	-	378	-	378
IT costs	-	-	-	4,149	-	4,149
Printing, postage and stationery	-	-	-	453	-	453
Advocacy	-	-	-	3,762	-	3,762
Sundry expenses	-	-	-	1,827	-	1,827
Research and development	-	-	-	9,793	-	9,793
Support work	-	-	-	19,109	-	19,109
Volunteer expenses	-	-	-	69	-	69
Customer entertaining	-	-	-	19	-	19
Accountancy fees	-	-	-	-	4,895	4,895
Governance costs	-	-	-	-	1,320	1,320
Legal and professional fees	-	-	-	1,932	60	1,992
HR support	-	-	-	-	2,593	2,593
Bank charges	-	-	-	26	-	26
	112,114	42,792	377	287,648	8,868	451,799

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

7 Grantmaking

	Grants to institutions	Grants to individuals
	£	£
Grants	107,335	4,779

The support costs associated with grant making are £0.

8 Grants to institutions

Name of Institution	Activity	£
Beefy's Charitable	Grants	43,000
Battle Batten- gene therapy	Grants	44,327
International disease registry project	Grants	12,774
Drug discovery in Zebrafish model	Grants	7,234
		107,335

9 Governance costs

	Unrestricted Funds	Restricted Funds	Total Funds 2016	Total Funds 2015
	£	£	£	£
Accountancy fees	4,895	-	4,895	5,829
Legal and professional costs	60	-	60	-
Governance costs	1,320	-	1,320	2,822
HR Support	2,593	-	2,593	2,128
	8,868	-	8,868	10,779

10 Trustees' remuneration and expenses

The trustees were reimbursed £1,320 (2015:£2,822) for travel and fundraising costs during the year. The trustees were not paid any remuneration during the year (2015: £nil).

11 Employees' remuneration

The aggregate payroll costs of these persons were as follows:

	2016	2015
	£	£
Wages and salaries	147,493	138,442
Social security	11,387	13,866
Other pension costs	2,219	-
	161,099	152,308

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

12 Debtors

	2016	2015
	£	£
Other debtors	-	168
Prepayments and accrued income	6,511	6,270
	6,511	6,438

13 Creditors: Amounts falling due within one year

	2016	2015
	£	£
Bank loans and overdrafts	-	2,670
Trade creditors	7,918	7,584
Taxation and social security	3,523	3,863
Other creditors	2,703	-
Accruals and deferred income	899	11,758
	15,043	25,875

14 Pension scheme

Defined contribution pension scheme

The charity operates a defined contribution pension scheme. The pension cost charge for the period represents contributions payable by charity to the scheme and amounted to £2,219 (2015 - £nil).

Contributions totalling £694 (2015 - £nil) were payable to the scheme at the end of the period and are included in creditors.

Batten Disease Family Association
Notes to the Financial Statements for the Year Ended 31 August 2016

15 **Related parties**

Controlling entity

The charity is controlled by the trustees.

16 **Analysis of funds**

	At 1				At 31 August 2016
	September 2015	Incoming Resources	Resources expended	Transfers	
	£	£	£	£	
General Funds					
Unrestricted income fund	129,004	240,153	(331,532)	5,115	42,740
Restricted Funds					
Gerri Lindup Effies Fund	(2,164)	-	-	2,164	-
St James Place	1,030	-	-	(1,030)	-
Cutler Trust	1,000	-	(1,000)	-	-
Roald Dahl	-	500	(500)	-	-
James Toohey	4,684	-	-	(4,684)	-
Sylvia Kapp Legacy	5,000	-	(5,000)	-	-
Isabel Sparkle	2,849	10,058	(11,125)	-	-
Euro Ed Project	6,113	3,086	(9,198)	-	-
Battle Batten CLN5	73,148	718	(17,570)	-	56,296
BioMarin	(6,514)	-	6,309	-	-
Beefys Charity Foundation	13,016	40,000	(43,000)	-	10,016
In Memory of Katie Freeman	(1,946)	852	58	1,036	-
In Memory of Melvyn Hancock	189	-	-	(189)	-
Clinical Nurse Specialist Funding	54,283	-	(32,190)	-	22,093
Ascot Research Funding	1,376	-	-	(1,376)	-
LINCL Research	1,196	-	(160)	(1,036)	-
BAT Cure	-	29,046	(3,047)	-	25,999
CLN6 Research	-	3,844	(3,844)	-	-
Oliviace CLN3	-	249	-	-	-
	<u>153,260</u>	<u>88,353</u>	<u>(120,267)</u>	<u>(5,115)</u>	<u>116,231</u>
	<u>282,263</u>	<u>328,506</u>	<u>(451,799)</u>	<u>-</u>	<u>158,971</u>

17 **Net assets by fund**

	Unrestricted	Restricted	Total Funds	Total Funds
	Funds	Funds	2016	2015
	£	£	£	£
Current assets	57,783	116,231	174,014	308,138
Creditors: Amounts falling due within one year	(15,043)	-	(15,043)	(25,875)
Net assets	<u>42,740</u>	<u>116,231</u>	<u>158,971</u>	<u>282,263</u>