

**Save Babies Through Screening Foundation UK**

**2010/11 Trustees Annual Report**

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## Statement from Pat Roberts, Chair



This has been our first full year as a UK Registered Charity and I am pleased to report on the very many achievements and successes we have had in driving forward our objects and goals during the year. This is largely due to the dedication of our Board of Trustees and our small team of volunteers, fundraisers and advisers, who give so selflessly of their professional skills and time. I would also like to take this opportunity to thank our Patron Jason Manford for his continued support. We are all drawn together with a common goal, to save the lives of children with rare genetic diseases.

Set against our successes in moving our work forward, this has also been a particularly difficult year for us all. During the year we have lost six children to Krabbes disease. These are the children of families who are an important part of our organisation. We know that there are other families in the United Kingdom that we are similarly affected, but that we do not know about. Our thoughts are with all families who have lost a child during the year.

As a small Charity we will face many challenges in the forthcoming year. We have many important projects that we are moving forward. This is against a backdrop of significant changes to the National Health Service, the impact of cuts to Government and Local Authority expenditure and the impacts of the current economic climate on members of the public who so generously support our fundraising.

Since Save Babies UK was established we have had considerable success, which of course brings with it real and valuable opportunities. However in light of the current economic situation it also brings the need for careful thought and planning on how we prioritise our work. I am so looking forward to progressing our projects and plans for the forthcoming year and most particularly, our plan to hold our first family conference in summer 2011.

## **Trustees Annual Report: 1 April 2010 – 31 March 2011**

Report of the Trustees for year ended 31 March 2011.

### **1. Reference and Administration Details**

Charity Name: Save Babies Through Screening Foundation UK  
(Save Babies UK)

Registered Charity Number, England & Wales: 1133080  
Registered Charity Number, Scotland: SC042501

Registered Address: 22 Bransdale Avenue,  
Guiseley,  
Leeds, LS20 8QA

Telephone: 01943 878770

Website: [www.savebabiesuk.org](http://www.savebabiesuk.org)

### **Board of Trustees**

Pat Roberts	Chair
Melanie McPherson	Vice-Chair
Janet Mills	Treasurer
Nicola Dolman	Secretary
Sam Hainsworth	

## **2. Structure, Governance and Management**

Save Babies Through Screening Foundation UK (Save Babies UK) is a non-profit making, charitable organisation, run entirely by volunteers. Our mission is to improve and save the lives of children by working to prevent disabilities or early death resulting from diseases that are both detectable and treatable at birth through newborn screening.

We are also working to advance the health and wellbeing of children suffering from Krabbes Leukodystrophy and other genetically inherited rare diseases in the UK. We aim to promote the advancement of research and treatments for these diseases whilst providing support for families and children diagnosed with Krabbes disease.

Save Babies through Screening Foundation UK is a charitable trust that is managed by a Board of Trustees operating through a Trust Deed which was enacted on 7<sup>th</sup> November 2009. Save Babies UK became a registered charity in England and Wales on 3<sup>rd</sup> December 2009. We received Charitable Trust status in Scotland in August 2011.

### **Structure**

Save Babies UK is a volunteer-run charity led by the Board of Trustees. Strategy is set and decisions are taken by the Board of Trustees.

### **Appointment of Trustees**

Trustees are appointed to the Board of Trustees on the basis of skills, knowledge and experience needed to run the charity successfully. The current trustees are the founding trustees.

All trustees give their time voluntary and received no remuneration or other benefits.

Further Trustees can be appointed by the Board of Trustees as required. Prospective trustees will be asked to provide information regarding their skills and suitability for the role of a Trustee. Training and induction will be provided for any further Trustees appointed, including guidance on the role of a Trustee.

### **Trustee Meetings**

The Trustees had two full Board meetings this year, in May 2010 and November 2010. At the November meeting the Board undertook an informal effectiveness review and considered the three-year strategic plan for Save Babies UK.

This year the Trustees have also initiated a programme of regular teleconferences throughout the year in order to progress arising business and further work underway.

### **Risk**

The Trustees manage an Assessment of Risk document and this is reviewed annually as a minimum. Where appropriate, systems or procedures have been established to

mitigate the risks the charity faces. Children and Vulnerable adults, Conflict of Interest and Volunteer policies are in place.

In addition to these activities, risk assessments are undertaken for any events undertaken.

## **Financial Review**

Our main source of funds this year has been from fundraising activities and donation.

We allocate 10% of all funds raised into the Jack MacPherson Family Support Fund in memory of Jack, the son of the Vice-president of the Board of Trustees. This is a designated fund to assist families in the UK with children suffering from Krabbe Leukodystrophy and other Leukodystrophies to purchase any necessary equipment or to meet other costs that are not provided by health services or Local Authorities.

We have also received a grant from Perkin Elmer to support the three year running costs of the PANS group, which is designated for the ongoing running of this group. Funds are held which are designated for the LSD Patient Collaborative UK clinical studies group.

In addition to designated funds, we aim to hold 1-years operating costs in reserve at bank, all of which is unrestricted funds.

The majority of other income will benefit the significant projects planned for 2011/12 and 2012/13.

## **Public Benefit Statement**

The activities benefit children in the UK by directly helping children and families of children with Krabbes Leukodystrophy through our support services.

Also, through raising awareness of the benefits of newborn screening with the public, government Ministers, the Department of Health and other health professionals and through supporting the research base for extending the newborn screening programme in the UK we can prevent disability, neurological difficulties and death in children.

Through forging working relationships with scientists, clinicians and other health professionals worldwide we are able to understand the position on research, clinical trials and treatments, in order to establish how this can benefit children in the UK and import better treatment.

The trustees confirm that they have complied with their duty to have due regard to the Charity Commission's guidance on public benefit in exercising their powers and in planning activities for the 10/11 year and future years.

### 3. Objects and Activities

#### Our Objects

Our Objects as set out in the Trust Deed are;

- To relieve sickness and preserve and protect the health of children suffering from Krabbes disease and Leukodystrophies and other genetically inherited diseases in the UK, in particular but not exclusively by funding research and promoting the extension of newborn screening;
- To preserve and protect the health of the families caring for children suffering from Krabbes disease and Leukodystrophies and other genetically inherited diseases in the UK by such means as the trustees may determine.

#### Our 10/11 objectives and activities

The objectives and main activities undertaken are as follows:

##### **1. To advance the programme of Newborn Screening in the UK**

Through the Patient Advocacy for Newborn Screening (PANS) working group we have:

- Embedded the PANS group as a fully operational group with clear objectives, a business plan and active membership.
- Identified and agreed through PANS the diseases for advocacy and submission work.
- Established and developed the stakeholder network for PANS engagement.
- Made a submission to Health Technology Assessment Research Group requesting a full review of newborn screening in the UK.

Through Save Babies UK we have:

- As a member of Rare Disease UK Prevention and Diagnosis Working Group, contributed to the report 'Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy' published in February 2011.
- Delivered presentations on Newborn Screening at the Child Health Conference in London in November 2010 and at a number of disease-specific conferences throughout the year.

##### **2. To provide support for children, and the families of children with Krabbes disease and other Leukodystrophies;**

- Provided support services for families with children diagnosed with Krabbes Leukodystrophy.
- Expanded knowledge of the support we can provide to families with health professionals in Children's hospitals.

**3. To promote and support research into Krabbes disease and other Leukodystrophies;**

- Engaged with scientists, clinicians and other health professionals both in the UK and overseas on the current position worldwide and in the UK, with a view to determining what is further required for the UK, including plans for a scientific workshop in 2012.

**4. To raise awareness of the work and objectives of Save Babies UK and further develop as an organisation;**

- Worked to expand awareness of Save Babies UK, newborn screening and Krabbes Leukodystrophy;
- Sought opportunities to expand our volunteer base;
- Continued to build our partnerships with other organisations and health professionals;
- Undertaken planning of three year strategic objectives and detailed business plans.

## 4. Achievements and Performance

This section expands on the activities that we have undertaken this year, with particular focus on what we have achieved through these activities.

### 1. To advance the programme of Newborn Screening in the UK

Save Babies UK has a collaborative approach to all our activities, in order to increase the opportunities available to us and avoid duplication of activities.

#### Patient Advocacy for Newborn Screening (PANS) working group

In December 2009 Save Babies UK established a UK working group collaborative – the Patient Advocacy for Newborn Screening (PANS) working group. This group is considering all the issues, risks and economic difficulties surrounding Newborn Screening (NBS) in the UK, both generic issues and those in relation to specific diseases. The group is Chaired by Save Babies UK and includes executives from Climb (Children Living with Inherited Metabolic Diseases), The MPS Society (Society for Mucopolysaccharide Diseases), the British Trust for the Myelin Project and a Pompe Family Support Practitioner. The group are advised and supported by health and diagnostic professionals.

The PANS working group is identifying, researching and putting forward proposals for those diseases that could meet the criteria for inclusion on the UK NBS programme and will make recommendations and arguments on those which are considered appropriate for submission to the National Screening Committee (NSC). These may not currently be on the horizon of the NSC or may be on a longer time track. PANS is encouraging the Department of Health to clarify and introduce more detailed plans for the extension of the newborn screening programme over the next 5 years. There has been considerable communication with MP's, Ministers and Department of Health staff throughout the year.

PANS has made a submission to the Health Technology Assessment Research Group outlining the issues and difficulties surrounding the Newborn Screening programme, including expansion and requesting a full review of Newborn Screening in the UK. This has been received and acknowledged although the process for getting this considered is likely to take up to 18 months.

PANS has continued to engage with different groups of stakeholders both internationally and in the UK. This has included sharing best practice for supporting extension of the screening programmes and requesting screening laboratory heads to hold Newborn Screening awareness days in their hospitals to support Newborn Screening Month in September 2010 and the mother and baby theme to National Pathology Week in November 2010.

To ensure that this work of PANS is accessible and transparent we commenced work on a dedicated website that was subsequently launched in May 2011.

#### Rare Disease UK

Save Babies UK is a member of Rare Disease UK and sits on their Prevention and Diagnosis working group. Throughout 2010 the group has been working on all

aspects of the prevention and diagnosis of rare diseases. The outcome of this work, the report "Improving Lives, Optimising Resources: A Vision for the UK Rare Disease Strategy" was published in February 2011 to coincide with World Rare Disease Day. This report will help inform the Government and Department of Health on rare disease planning. It is recommended that all EU Member States have rare disease plans in place by 2013.

#### Other Activities

- In June 2010, Save Babies UK gave the keynote speech at the Perkin Elmer World Newborn Screening Conference in Finland. In addition to raising awareness of the importance of Newborn Screening and the UK position, this allowed us to benefit from listening to experts from around the world speaking on how newborn screening programmes are being extended and implemented.
- Save Babies UK were invited to speak about Newborn Screening at the following conferences: The LSD Family Conference in Edinburgh in September 2010, The 2010 Child Health Conference in London, the AGSD (Pompe Disease) Family Conference in November 2010, the Perkin Elmer Conference for UK Laboratory professionals in November 2010 and the USA LSD Transitional Research Network Meeting in Las Vegas in February 2011.

## **2. To provide support for children, and the families of children with Krabbes disease and other Leukodystrophies**

Save Babies UK provides an information and support network for families with children diagnosed with Krabbes Leukodystrophy. This is a rare genetic disease and we believe there are three elements to the successful delivery of this service:

1. Fast referral to the support provided by Save Babies UK: we have continued to network with health professionals in Children's hospitals across the UK to raise awareness of our organisation and the support we can provide.
2. Interactions with families being personal, well informed and responsive to the needs of the family: our volunteers that provide this support have firsthand experience of Krabbes disease.
3. Financial support where needed: we consider and where possible provide financial support for specific items for children where this is required but not provided by the NHS or the Local Authority. We have a designated fund for this activity.

Throughout the 2010/11 we were made aware of 6 children in the UK diagnosed with Krabbes disease. We were able to offer support to the families who contacted Save Babies UK, by providing information, helping families access the services available to them and through emotional support. We were able to make grants for equipment and travelling expenses from our dedicated fund. We will continue to develop and improve this service as needed in response to feedback from families.

## **3. To promote and support research into Krabbes disease and other Leukodystrophies for development of better treatments for these diseases**

Save Babies UK is a member of the Lysosomal Storage Disorder (LSD) Patient Organisation Collaborative. Krabbe Leukodystrophy is one of the approximately 40 LSDs, which are rare inherited metabolic disorders. The group meets quarterly to discuss all aspect in relation to LSD and to plan activities and work on behalf of LSD patients and their families in the UK.

As part of the LSD Patient Organisation Collaborative, Save Babies UK continues to support a UK clinical studies group in inherited metabolic disorders, for a three year period. This group working with the Medicines for Children Research Network, is Chaired by a consultant in Inherited Metabolic Disease at Birmingham Children's Hospital. The group are developing a comprehensive and collaborative prioritised research programme in inherited metabolic disease which will permit the investigation of important diseases affecting children, improving both knowledge and quality of care. The Executive Chair of Save Babies UK has taken up membership of this clinical studies group, representing the LSD Collaborative.

The LSD Patient Organisation Collaborative held their first Conference for LSD families in Scotland in September 2010. LSD Patients had the opportunity to meet with families similarly affected by these rare diseases and to hear presentations from scientists and clinicians on the developments in research, clinical trials and treatments for particular LSD's. Feedback from families involved has been that they found the conference to be extremely valuable.

Save Babies UK, as part of the LSD Patient Organisation Collaborative has been working on a Department of Health funded research project examining the current transition processes when a child moves from paediatric to adult health services. The aim is to review and evaluate how transition has previously been managed and to make recommendations on how the services can be improved or developed. This project is being carried out in 5 stages and is due to complete in December 2012.

#### **4. To raise awareness of the work and aims of Save Babies UK and further develop as an organization.**

##### Charity Development

As part of our strategy development to establish how we can progress our objectives most effectively and develop as an organisation to achieve this we continue to develop a wide network with professionals internationally who specialise in Newborn Screening, Leukodystrophies, Lysosomal disorders and other rare genetic diseases. Save Babies UK Executive Chair was sponsored to attend the LSD World Symposium in February 2011 in the USA, a significant learning opportunity in achieving the understanding to move the work forward.

We undertook an informal Board effectiveness review in November 2010 and undertaken planning of our three-year strategic objectives and detailed annual business plans.

We have continued to build a network of volunteers and supporters who give generously of their time to help us achieve our objectives.

## Raising Awareness

We have raised awareness through the following channels;

- Continuing to provide information and updates on the work of the charity and developments in interest fields via our website;
- Development of information materials for families and research/health professionals;
- Providing information at our fundraising events;
- Presentations at health and patient conferences;
- Through collaborative relationships with other patient advocate groups.

## Fundraising

Save Babies UK is a charitable organisation that receives no government funding or grants. The success of our work is dependent on the generosity of people in making donations and supporting our fundraising events.

To support our objectives and activities through the year, Save Babies volunteers have held collections, sponsored events and fundraising events. We have also received charitable donations from individuals and organisations. We would like to thank all those who have given generously and have been involved in raising funds to allow the continuation of our work.

## 5. 2011/12 Activities

Through the PANS group we will continue to build a stronger voice to contribute to national policy development, through assessing and presenting the evidence base for extending the Newborn Screening Programme and promoting consistency among nation countries. We will pay specific attention to the area of economic evaluation in presenting our proposals. We will increase public awareness of diseases that are detectable at birth and the importance of newborn screening in terms of preventing disability and saving lives. We will develop information materials to assist with our aim.

Our main project on Newborn Screening, which is currently in feasibility is to hold a 'Newborn Screening Saved My Life Day' at Westminster, celebrating the children whose lives have already been saved through the UK newborn screening programme and raising awareness of how much more good can be done by extending the programme to cover additional disorders. We will also launch and publish our dedicated PANS website to ensure information on our work is freely available.

We will continue to build and strengthen collaborative working relationships, ensuring shared resources and activities where it can support our aims. We will attend the 7<sup>th</sup> regional meeting of the International Society for Neonatal Screening in 2011 with the benefit of a Travel Scholarship. Following on from the success of the LSD Collaborative conference for families in Scotland the LSD group is now looking at the feasibility of something similar in Northern Ireland.

We will consider and explore further opportunities to support research into Leukodystrophies. We are currently planning for a scientific conference on Krabbes disease to be held in the UK in Autumn/Winter 2012. This will further consider the current position for the UK and associated issues, what can be done to save lives and improve the quality of life for children with Krabbes, the resources that are required for this and where funding needs to be directed. Planning and fundraising to deliver the conference has already commenced.

We will continue to develop the family support we provide, monitoring the effectiveness of the support and examining how we can provide further support services to a greater number of families. We will extend our campaign to generate greater awareness of Save Babies UK among healthcare professionals in children's hospitals, in hospices and care services to enable families with children that have been newly diagnosed with Leukodystrophies to seek help if needed. We will hold a family conference for Krabbe families in the Summer of 2012 and if successful we hope that this will become an annual event.

To build on the growth and momentum that we have achieved as an organisation we aim to expand our volunteer base, through maintaining our existing supporters and recruiting further volunteers. We will launch our e Newsletter in the Spring of 2011 to keep our supporters informed of progress and engaged in our work. We will actively seek to apply for grants and increase our fundraising activities to support the activities outlined above.

## 6. Declaration

The trustees declare that they have approved the trustees' report above.

Signed on behalf of the Charity's trustees

Signature Patricia A Roberts

Full Name Patricia Ann Roberts

Position Executive Director and Chair

Date 25<sup>th</sup> November 2011