MPS

Summer 2012

In this edition…

Celebrating **30 years of the MPS Society**

Your stories from **MPS Awareness Day,** 15 May 2012

The launch of the MPS Society's new fundraising initiative Wicked Genes

Society for Mucopolysaccharide Diseases

mps

Please donate online
www.mpssociety.co.uk,
phone 0845 389 9901,
text MPSS01 £2/£5/£10 to 70070
or post your donation
to our office, MPS House.

The MPS Society

Founded in 1982, the Society for Mucopolysaccharide Diseases (the MPS Society) is the only national charity specialising in MPS and Related Diseases in the UK, representing and supporting affected children and adults, their families, carers and professionals. The MPS Society:

Acts as a **support network** for those affected by MPS and Related Diseases

Brings about more **public awareness** of MPS and Related Diseases

Promotes and supports **research** into MPS and Related Diseases

MPS & Related Diseases

Mucopolysaccharide (MPS) and Related Diseases affect 1:25,000 live births in the United Kingdom. One baby born every eight days in the UK is diagnosed with an MPS or Related Disease.

These multi-organ storage diseases cause progressive physical disability and in many cases, severe degenerative mental deterioration resulting in death in childhood.

At present there is no cure for these devastating diseases, only treatment for the symptoms as they arise.

Where does your money go?

A donation of *£2 per month* could help us to offer so much more support in so many ways:

Access to clinical management and palliative care

MPS Regional Specialist clinics

Support with disability benefits

Paving a child's way in accessing education

Upholding rights in employment

Advising on home adaptations

Bereavement support

Front cover photo: Louise Baker and Andrea Parker who ran the London Marathon 2012 for the MPS Society. See our Fundraising section for their full story.

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Magazine Deadlines

Autumn	1 Sep 2012	Winter	1 Dec 2012
Spring	1 Mar 2013	Summer	1 Jun 2013

To submit content email **magazine@mpssociety.co.uk**

IN THIS ISSUE…

Summer 2012

www.wickedgenes.co.uk

Welcome to the Summer 2012 MPS Magazine featuring stories from our members, sections on governance, our unique advocacy support service and events plus the latest updates on research and therapies.

In this edition we are delighted to celebrate the Society's 30th anniversary year including our appeal on Justgiving.com and our Reception at the Palace of Westminster. We are also featuring your photos and stories from MPS Awareness Day, 15 May 2012.

If you have any material to submit, please email magazine@mpssociety.co.uk or phone 0845 389 9901. We would love to hear from you! In the meantime, enjoy the magazine!

Once you have read this MPS Magazine, please pass it on to your family, friends and colleagues. Help us spread the word about MPS and related diseases and the work we do. www.mpssociety.co.uk

Message from the Chief Executive

www.wickedgenes.co.uk

In the Spring MPS Magazine I eluded to the funding challenges facing the MPS Society and its work behind the scenes to develop a new way for people to raise money to enable the MPS Society to continue to fund vital research and extend its advocacy and support programme. I am delighted to announce the launch of **Wicked Genes**. You can read more about Wicked Genes further on in this MPS Magazine.

Wicked Genes aims to appeal to a wide range of people. Whilst the MPS Society is well known to those affected, it is still a relatively small charity. A small charity with big ideas though. Wicked Genes is being promoted across the UK to schools, universities and businesses as a new exciting and fun way to get groups of people connected to raise money for a very very worthwhile cause. We know that many organisations will get creative and come up with wicked fundraising ideas and we are waiting eagerly to get involved. We are fortunate enough to have experts up and down the country ready and willing to come along to schools and businesses to share their own personal stories of MPS within their family, which will really bring home how important this is.

Wicked Genes is about having wicked fun, doing something wickedly crazy, or wickedly daring, or wickedly silly, or wickedly lovely. It's about coming together with friends, workmates and strangers to raise a bit of money to save children's lives.

We sincerely hope that by widening the MPS Society supporter base and encouraging schools, universities and businesses to organise a Wicked Genes event that we will be able to raise the money urgently needed to fund the pioneering Genistein clinical trial and much more.

There is a Genistein Clinical Trial for Sanfilippo disease (MPS III A, B, C) that may show benefit to other MPS diseases including MPS I, II and VI. The MPS Stem Cell Group and Manchester Children's Hospital are ready to start once the MPS Society has raised the £800,000 needed.

Further on in the MPS magazine you will read the exciting news from the MPS Stem Cell Group on their Gene Therapy work in MPS I and MPS III. We now need to move on from curing the Sanfilippo mice and raise the funds to run a Phase 1/2 gene therapy clinical trial in children with Sanfilippo disease.
For thirty years the treatment of choice for MPS I Hurler disease has been bone marrow transplant (BMT) and cord blood transplant (CBT) but the question now being asked is 'Can our younger children treated with BMT and CBT in their early months of life benefit from enzyme replacement therapy?'
You can help Wicked Genes be so successful that the MPS Society can launch a clinical trial to answer this question and provide vital research funding for Fabry disease, the Mucolipidoses and other related Lysosomal storage diseases?

Help us to get the four nations of the United Kingdom raising money for **Wicked Genes** so we really can create a future with hope for children with genetic diseases.

Please go to the Wicked Genes website **www.wickedgenes.co.uk** and download a fundraising pack. Please tell your friends and relatives to tell theirs all about Wicked Genes and ask schools, businesses and universities to raise funds. If a business, school or university would like a Wicked Genes speaker, please contact Laura Troll, Development Officer for Wicked Genes, by phone on 0845 389 9901 or by email wickedgenes@mpssociety.co.uk

The success of Wicked Genes will determine how quickly we can support vital research and provide even more practical help to children and individuals with MPS, Fabry and related diseases and their families.

Christine Lavery
Chief Executive

URGENT BREAKING NEWS – Has AGNSS had its day?

The National Specialist Commissioning Team (NSCT) and the Advisory Group for National Specialist Services (AGNSS) has over the last 7 years become a model for evidence-based evaluation and delivery of both drugs (Enzyme Replacement Therapy and the expert MPS/Fabry clinical services in London, Birmingham, Cambridge and Manchester. It is because of AGNSS that all our members in England who meet the clinical guidelines are treated with ERT.

Under the new NHS plans to be implemented on 1 April 2013 the MPS Society is now of the understanding that a form of 'AGNSS' will be found a place within the new Commissioning Board for managing MPS/Fabry clinical services. However crucially the funding of existing MPS therapies (ERT) and approval of new MPS therapies (ERT, Chaperone and Intrathecal) will be in the domain of the National Institute of Clinical Excellence (NICE) with some weighting of cost per Quality Adjusted Life Year (QALY) for rarity and judged against a policy of Value Based pricing (VBP).

This lack of certainty regarding the future of AGNSS and whether any new structure will protect services for our MPS and Fabry members is very worrying. The MPS Society is in no doubt as the situation is at this moment that we will need all the efforts of our members to fight to save a service that has served our members so well and can be considered 'a jewel in the crown of the NHS'.

We will be keeping in touch with you and advising you of how you can help over the coming weeks with contacting your MP and approaching media. Andrew Lansley might have seen off healthcare workers but he is not going to see of our members without a fight. **Christine Lavery** c.lavery@mpssociety.co.uk

News from the MPS Board of Trustees

The Society's Trustees meet regularly. Here is a summary of the main issues that were discussed and agreed at the Trustee Board Meeting held 27 - 28 January 2012.

Treasurer's Report
The Treasurer presented the accounts for year ending 31 October 2011. These were agreed subject to a few minor amendments in the Trustees' Report and the Financial Review.

Risk Management
Trustees were advised that the staff handbook has now been completed and approved by Peninsula. It was agreed to amend the risk register accordingly. The Chief Executive advised that the Disaster Recovery Plan and Succession Planning Strategy were works in progress.

Personnel
The Trustees re-affirmed their decision that the vacant Advocacy Officer post continue to be frozen until the funding for this financial year has been secured. The Chief Executive advised Trustees that Lindsey Wingate would be working three days a week until her leave date. A discussion on performance management took place and one trustee agreed to provide copy from her current employer.

Clinical Management
The Chief Executive through her report updated the Board on the progress of the QIDIS Transition Project which is in the writing up stage and confirmed that a meeting of the Advisory Transition Group had met. Trustees were advised of Dr Ramaswami's appointment as paediatric consultant in the LSD team at Manchester Children's Hospital and the appointment of Dr Chris Hendriksz as adult LSD clinician at Salford Royal Hospital, Manchester. The Trustees were advised of the on-going clinical management meetings that had taken place at the Royal Free Hospital, London and Great Ormond Street, London. Trustee were appraised of the challenges regarding a Welsh baby denied funding for a haematopoietic stem cell transplant at Manchester Children's Hospital.

Advocacy
It was reported that the Advocacy team have been very busy and are working on a number of complex issues with regard to access to appropriate adult care packages.

Association of British Pharmaceutical Industry (ABPI)
The Chief Executive reported on her meeting with Andrew Zaman at the ABPI to explain the MPS Society's considerable concerns that the ABPI make no effort to consult on the introduction of rules that may not be in the MPS members' interests. It would appear that the ABPI is a self-serving and conflicted organisation as it is governed and funded by major pharmaceutical companies in the top 100 FTSE. It was agreed that the Chief Executive write to the ABPI Chairman.

Relationships with the Pharma Industry
The Chief Executive and members of the Senior Management and Advocacy team continue to work collaboratively with the pharmaceutical industry in areas pertinent to the membership whilst retaining a high level of independence.

Fabry International Network (FIN)
The Chief Executive advised the Trustees of the resignation of Kees Bosman as Treasurer of the Fabry International Network.

MPS 30th Anniversary Appeal

This year we are delighted to celebrate the Society's 30th anniversary. Thirty years ago, our fight was just beginning.

Mucopolysaccharide (MPS) and Related Diseases were little known and a diagnosis usually brought families face to face with social isolation, fragmented clinical approaches to medical management and an almost certain death for those affected at an early age.

Today the UK MPS Society is a community of over 1200 families spread throughout the United Kingdom working in partnership with MPS Specialist Centres for Children and Adults, the Pharmaceutical Industry, Government and Regulators. Perhaps the MPS Society's greatest achievement has been to create a favourable environment in which to foster change for the benefit of children and adults living with these devastating conditions, their families and the professionals who support them.

Much has been achieved on the research, treatment and therapy front particularly over the past ten years. The work we do, however, is far from over. There are still so many goals to be conquered, not least for the MPS and Related Diseases for which there are still no therapies. For this, hope is the prime motivator.

As the UK MPS Society celebrates its 30th anniversary we need your support to enable our fight to continue. Donations are vital and will be used to support our advocacy service and benefit those affected by MPS and related diseases.

To contribute to our 30th Anniversary MPS Appeal please visit
www.justgiving.com/Society-for-Mucopolysaccharide-Diseases

Don't forget that you can subscribe to our MPS Magazine and order MPS merchandise, information materials and seasonal goods online at www.mpssociety.co.uk

News from the MPS office

Introducing **Martine Tilley**

Hi, my name is Martine Tilley and I joined the MPS Society as Office Administrator in May 2012.

My working background is within Administration and Customer Service spanning 13 years. I have worked for many different companies which has given me plenty of experience. I hope now to make a difference and support the Society and its Members.

Outside of work my family and I enjoy attending the British Superbike meeting held all over the country, we follow a friend's team in the Superstock 1000 league. When not attending them we enjoy going for a bike ride, cinema, theme parks or when I can the theatre.

I would like to thank everyone for making me feel very welcome and helping me settle in.
I am looking forward to future.
Martine Tilley m.tilley@mpssociety.co.uk

Introducing **Laura Troll**

Hi there, my name is Laura and I have recently taken on the role as Development Officer for Wicked Genes with the MPS Society.

I feel very privileged to have been given this opportunity to become involved with the MPS Society to launch the new fundraising initiative and am excited about the challenges ahead of me. It has been great meeting families, board members and the staff team here and I am so impressed, not to mention inspired with the dedication throughout.

My background has always involved working with vulnerable people individuals and families. My roles have been varied and include building Circles of Support, facilitating person centred planning and most recently, supporting teams who work with individuals to plan for the future and live typical lives within their communities in Western Australia.

I firmly believe in inclusion and that everyone has the right to live the life of their choosing with the supports they require. Over the last ten years I have lived in England, Scotland and Australia so have had the opportunity to work with a huge spectrum of people whom have only strengthened my belief in inclusion for all.
Laura Troll l.troll@mpssociety.co.uk

Farewell to **Fiona Hopson**

Well guys, it took a lot of contemplation and hesitance to come to the decision to leave MPS. My little boy starts school in September so I needed to find a job closer to home….

It was an extremely hard choice to make as I feel so attached to the MPS Society and everything it stands for and does for its members.

I have worked at the MPS Society for over two years and have been so proud to serve you all. I am continuing with a career in the children's charity sector. The MPS Society has given me so much, I have experienced far more working here and have gained so much more job satisfaction, than I ever did in any of my previous career paths!

I would like to say goodbye and best wishes to all the MPS Families, Supporters, Medical Professionals, Staff and Trustees at MPS Society… You all do such an awesome job.
Thank you from the bottom of my heart.
Fi Hopson

WHAT’S ON!

SPECIAL EVENTS

Childhood Wood Remembrance Day: 15 July 2012

Alton Towers Family Weekend: 14 – 16 September 2012

We have a few places still available.

Download a booking form from the MPS website www.mpssociety.co.uk

Childhood Wood Planting Day: 21 October 2012

MPS REGIONAL CLINICS

Manchester BMT clinic: 13 July, 20 July, 5 October, 19 October

Birmingham MPS clinic: 23 November

Adult Birmingham Fabry clinic: 18 September, 16 October

Northern Ireland clinic: December (date tbc)

MPS Society continues to support those affected by Metachromatic Leukodystrophy

The Society for Mucopolysaccharide Diseases continues to welcome members with Metachromatic Leukodystrophy (MLD).
We invite anyone who is affected by this disease, or professionals working with those affected, to contact us.

We have an Advocacy Support Officer who supports individuals and their families affected by MLD and so this does not affect the current service already provided by the MPS Advocacy Team to those affected by MPS and Related Diseases.

We have developed a Guide to Understanding MLD fact sheet which is available from the MPS office, and downloadable from the MPS website, plus a range of other information resources which cover issues related to those affected and their carers.

For further information please contact the MPS Advocacy Team by phone on **0845 389 9901** or email **advocacy@mpssociety.co.uk**

Reduce paper consumption!
We need your help...

To cut down on postage and paper so we can put more of our resources into helping our members, we would like to become as paperless as possible.

If you would like to be informed by email of our events and activities, please email mps@mpssociety.co.uk with your current email address.

We will add this to our database purely to keep you informed and will not pass your details on to any third party.

We understand that this may not be possible or convenient for everyone and if, at any time, you feel you would rather receive information by post or be removed entirely from our mailing list you can email the above address or call 0845 389 9901.

ANNOUNCEMENTS

Dr Jane Collins

Dr Jane Collins, Chief Executive of Great Ormond Street Hospital, has announced she is to stand down to take up the appointment of Chief Executive at Marie Curie Cancer Care. Dr Collins, despite other significant demands on her time, has over the last two years worked with the MPS Society and Gaucher Association to improve the focus of Lysosomal Storage Diseases at Great Ormond Street and helped to improve the co-ordination of appointment schedules and out of hours services for our members. I for one will always be grateful to Dr Collins for her support and wish her well in her new post.
Christine Lavery c.lavery@mpssociety.co.uk

New members

Mr and Mrs Shepherd have recently been in contact with the Society. Their daughter Anabelle has a diagnosis of Hurler disease. Anabelle is one year old and the family live in Portsmouth.

Silvia and Lyndon have recently been in contact with the Society. Their son Maurice has a diagnosis of Hurler disease. Maurice is 7 months old and the family live in the South East. They would be happy to be contactable with other families that have children with a similar condition.

Lisa has recently been in contact with the Society. She has a diagnosis of Fabry disease. The family live in the Yorkshire area.

Mrs K Clayton has recently been in contact with the Society. Her daughter Lily-May has a diagnosis of Morquio disease. Lily-May is 10 years old. The family live in the East.

Mr Bukhari has recently been in contact with the Society. His son Aydin has a diagnosis of Morquio disease. Aydin is three years old and the family live in the North West.

Ms G Ahmed has recently been in contact with the Society. Her son Haris has a diagnosis of Sanfilippo Type A. Haris is four years old and the family live in the North West.

Ms Pascoe has recently been in contact with the Society. Beau has a diagnosis of Sanfilippo disease. Beau is 18 months old and the family live in the East Midlands.

Deaths

We wish to extend our deepest sympathies to the family and friends of:

Landon Owen who suffered from Sanfilippo disease and who died on 16 April 2012 aged 14 years.

Jack Atkinson who suffered from Hunter disease and who died on 27 April 2012 aged 13 years.

Alice Marston-Taylor who suffered from Sanfilippo disease and who died on 24 May 2012 aged 24 years.

Craig McLean who suffered from Sanfilippo disease and who died on 28 May 2012 aged 18 years.

In memory

Colin Arrowsmith

29 February 1980 – 8 February 2012

My heart broke on that sad day
When my gorgeous big brother passed away.
He was kind and caring, a really special man
Newcastle United's greatest fan.
He loved his family with all his heart
Hated it most when we had to part.
Had fights and arguments like all good siblings do
But underneath I loved him – I knew he loved me too
Negativity and conflict he could not stand
Why can't people get on? He just didn't understand.
He had so many friends and I know why
He really was a truly remarkable guy.
He protected me when I was small
Just being his sister makes me proudest of all.
I miss my brother too much to say
A pain like this doesn't go away.
I will love you Colin until my very last breath
A love this big won't be ended by death.
Love Claire

It's vivid in my memory, my greatest day of all
The day you came in the world I floated 10 feet tall.
'Your little son has Hunter's – that's what we were told.
The news that day broke our hearts, you weren't very old.
All the dreams I had for you were shattered on that day
All my hopes for you were gone, the sky turned to grey.
I loved the time I spent with you, I cherished every day
You were a pleasure to be around, my memories won't fade away.
My heart was heavy, my pain was great
My pride in you too large to state.
My longest night, my saddest day
My precious son, you slipped away.
I miss you my son, with all my heart,
You live on in my memories – we will never part.
Love Mam and Dad

Georgia Lewis

11 July 1995 – 19 February 2012

Our beautiful Georgie Pie, our "Little miss sunshine", passed away very suddenly in February, aged 16 years.

It was a big shock and adjusting to life without our precious daughter is incredibly hard. We're trying to focus on all the wonderful memories that we have of Georgia, her zest for life and her beautiful, infectious smile and giggles that will stay in our minds forever. Below is the 'letter to Georgia', that I wrote and read at her funeral.

We love and miss you so much Georgie Pie, all our love from mum, dad & Chrissy…………

To our Dear Georgie Pie,
Our 'Little Miss Sunshine'!

Just a little note to let you know how much we miss you & love you, but I think you already know that, as we feel you watching over us, still with us, guiding us.

Everyone here today will have their own special memories of you. Even people across the globe who are not here, you have touched their hearts forever. What an incredible achievement that is!

Our life together was certainly fun, unique and often very challenging. 'Never a dull moment' as your wonderful grandma often used to say! When you were younger, your cheeky character, mischievouness and wicked sense of humour would often get us into all sorts of predicaments! You were incredibly strong willed and knew exactly what you did and didn't want!

Because of you, we were able to experience 'once in a lifetime' opportunities – like our time at 'Highgrove', with Prince Charles and Camilla – as we all sat talking, you watched him intently, not taking your eyes off him, but we KNEW you were just focussed on his ears and were willing you NOT to grab them! Also, during our visit to 'Downing Street', we were hoping and praying that you wouldn't start singing 'Postman Pat' at the top of your voice in front of all the TV reporters!

Talking of 'Postman Pat', which was one of your favourite videos to watch (over and over and over again!), you thought that every little old lady with grey hair and glasses was either 'Mrs Goggins' or 'Granny Dryden' – try explaining that to complete strangers when you insisted on shouting out and calling them, wherever we were!

You never did things by half. Your pure innocence and sense of fun certainly kept us on our toes! One incident which we remember vividly (and have video footage to prove it!) was in 'Disneyworld, Florida' – surprisingly, you patiently waited your turn to see the Disney character 'Goofy', then promptly hit him across the nose! He certainly wasn't expecting that! But we weren't surprised! We learnt to expect the unexpected with you and nothing ever phased us!

There are SO many wonderfully funny and precious moments that we could recall, and I could stand here all day and talk about so many aspects of your life, they will stay with us forever, we will cherish those memories now and keep them alive. When you were born, we dreamed of all the things we would teach you over the years – little did we know you were sent to teach us. We've learnt that old saying of 'Life's too short' was never more meaningful – it became our rule to live by and we strived to ensure you had the best life possible.

I wonder if you'll ever know just how much you enriched our lives, and that of everyone who knew and loved you. You taught us to be compasssionate, patient, thankful, to enjoy life and to never, ever take anything or anyone for granted. You gave us unconditional love and made us realise how precious life is. You taught us that the simple things in life really are the best – just a smile, laughter, a kiss or a hug are just priceless.

You are such a special, inspirational, beautiful young girl, unique in every sense, one in a million. We will miss your wicked sense of humour, your contagious giggles, your big cuddles and holding hands, your stunning 'chocolate' brown eyes that spoke volumes and your beautiful smile that would light up a room and just melt our hearts.

Georgie, you were so tired and we know you are at peace now – surrounded by those who love you in heaven. The angels have the privelege of caring for you now.

Goodnight, god bless, sweet angel.
We will love and miss you forever.

With all our love from,
Mum, dad, Chrissy and all your family and friends who love you so dearly.

Dean Doherty

2 July 1999 – 25 February 2012

Someone Special

Someone touched my soul
today and made me
realise that we
don't have to say
a word when love
speaks in our eyes

Because there's
such a feeling,
a truth that never lies.
Unspoken words say
more somehow when
whispered by the eye.

Gold medal for Chris!

Hello to all you readers! My name is Chris Isaac and I have Morquio – I live independently in a flat in Bridgwater, a few miles away from where I grew up with my brother and sister and parents.

My favourite sport at school was table tennis and I used to love playing in the school teams. Since then I haven't really played (15 years ago – I'm now 31!). More recently I have joined the DSAUK (Dwarf Sports Association) where I have been playing other competitors who have the same arm spans as me. I play in my electric wheelchair and usually my brother or sister come with me for the weekend event to see me play and to meet and socialise with all the other little people.

Last May I am proud to say I won a gold medal at the Dwarf Sports Association held annually in Walsall. Previously I had gained a silver medal. I am really proud of my medal and this coming May I plan to compete again and defend my gold medal. **Chris Isaac**

Update from Sarah

Hello again,

A lot has happened in the past few months. The amounts of coursework and the level of work expected from me in my Open University degree have increased. This means I have to be more dedicated in managing my time so as to fit this around work. It was tricky to start with but it is getting easier. I do have to plan my work far enough ahead of schedule though, so as I am not late with my deadlines if I was to have a bad few weeks in terms of Fabry's. It certainly isn't easy to write an essay on brain development when you are feeling unwell.

I plucked up the courage to tell my best friend about Fabry's last month. I have known him for years but never brought myself to tell him, it never seemed to be the right time. I always get worried about how other people will react and whether they will treat you differently. But I don't know what I was worried about, he was fine about it, and has been so supportive offering to come with me to appointments to keep me company. It makes me wonder why I never told him before; I am so glad he knows now though because it makes things easier and has brought us closer together.

I have also decided that I want a change in my career, at the moment I am only working part time in a shop, which suited me fine until the company had to bring in cut backs. It's affecting everyone I know but it's difficult surviving on only a few hours a week on minimum wage when I feel up to working more. I have applied for a few new jobs and have a couple of interviews lined up so fingers crossed!

Another thing that has happened this month is that my mum and I were lucky enough to be invited to the Houses of Parliament to celebrate the MPS' 30th birthday. We were up and into London by 11am which meant there was time for some shopping and exploring Oxford Street! Well you can't go to the capital and not shop! The trouble was the weather was so hot! We can't complain about the nice weather I know, but the heat tends to bring on painful hands and feet which aren't so nice walking round a big city, so we found a shaded spot along the Thames to wait until we were allowed into the Houses of Parliament. When we got there we got shown to a room which was situated along the Thames, so we were able to sample some of the food and drinks in the sun whilst catching up with some people we had met before.

It is always so nice to meet people like me at these meetings, to catch up with people you only get to see at functions like this and to hear people's stories; I feel I speak for all of us when I say it is nice to feel like you are not alone in what you are going through. We were also lucky enough to be joined by the Secretary of State for Culture, Jeremy Hunt. He was genuinely nice and the way he spoke to everyone was inspiring, He said a few words and then I was lucky enough to grab a photo with him and Christine Lavery, who I think everyone would be behind me in thanking her and all of the MPS team for all their hard work and support over past years.
Here's to the next thirty years!
Sarah

Get in touch… If you would like to share your story and photos in the MPS Magazine please write to us, phone **0845 389 9901** or email **magazine@mpssociety.co.uk**

On a Hunter's journey...
Deciding on a decision!

June 16th 2012 will mark two years since our beautiful baby boy received a diagnosis which changed our life. Looking back to that fateful day, its amazing just how quickly time flies, life changes, and our MPS II Hunter boy has surely changed too for the better but, so have we. Since starting school in September 2011 Caleb has achieved so much. Settling into school, becoming completely toilet trained during the day and throughout the night, and refining his already artistic flair for drawing faces everywhere! Including on sofas, walls and tables!

It is fair to say that the first year of diagnosis is the most hardest to tackle. I lost myself, I lost some friends, but I gained so much knowledge about MPS and how being positive makes a massive difference. The second year is when I believe you start to feel a little more normal. All the things that used to worry us has become meaningless, we are now forced to channel our energies into ensuring our son gets better.

In the last update I spoke about our journey becoming independent with Caleb's weekly Elaprase infusions. This is now second nature to us. It's been a year since we became independent. We can now complete each weekly infusion with little or no issues, Caleb accepts that this is life and in turn this prompts Mark and I to also accept that this procedure is normal life for all of us.

One of the biggest changes to our life happened in January this year - The Hunter Intrathecal Trial. This break through clinical trial had been making waves in the USA with Hunter families apparently queuing up to get a chance to be part of it.

After many talks with the doctors and the trial team we decided that we would put Caleb through the rigorous testing process with the hope of him qualifying to be part of something quite exciting and potentially beneficial.

Caleb wasn't even back from theatre when we received the news from the trial team that the first hurdle was done, and Caleb had passed the first stage after his MRI and Lumbar Punch. In order for Caleb to be eligible his brain pressure had to fall below 30. The nurse came back to ward 10 with the news that his brain pressure was good, we knew the next decision would be life changing. Caleb's result was then put into a database for randomization. What seemed like hours, was in reality minutes, and our doctor presented us with the news we had been anticipating. Caleb randomized to receive the drug. We were in. No ifs or buts. Life would change from now dependent on our decision.

The trial would almost certainly take up 7 full days of our life each month, we live in Hertfordshire, and the trial is based 2 and a half hours car journey away in Birmingham. We decided in a nano second we wanted to go through with it. Not even thinking about the implications, financial pressures and long periods away from home and how all of these things would affect us all. Six months has flown by, and we have taken the trial for what it really is, a safety study in getting Elaprase to the brain through an intrathecal port.

It is not a cure or a miracle drug, and this is something you have to really understand when deciding to take part in a clinical trial drug testing. It is a risky choice, time consuming, tiring, emotional and worrying. But at the same time we experience moments of joy, elation and hope for the future, all of which outweigh some of the risk taken. We are so proud that our son is changing medical text books and is able to be part of scientific medical research which could quite possibly help those diagnosed in the future. Some would even question why we could even consider this if it puts our beautiful boy at risk and the reality is, our boy is at risk, so choice for us was not an option. We just had to decide on a decision! The disease is what the disease is.

We know that at some stage our boy will start to change, decline, and possibly worsen. This drug has so many possibilities, he may not decline as quickly, there is much hope, especially when we consider what normal ERT does for Caleb. The only way we really know for sure that we are doing the right thing is our boy's smile. Caleb tolerates the long trips to Birmingham so well, he is the same happy little boy he has always been, thriving at school, challenging at home, and compliant for all his medical appointments. He constantly smiles, and will always let us and the trial team know when enough is enough. And that is all the reassurance we need in knowing we made the best decision for our beautiful boy Caleb. **Daniella Vandepeer**

MPS Regional Specialist Clinic Programme

The MPS regional clinic programme involves centres including Manchester, Belfast, Birmingham, Bristol, Cardiff, London and Newcastle. The programme aims to enable individuals affected by MPS and Related Diseases to access a consultation with a tertiary centre consultant without having long distances to travel to their designated specialist centre. The clinics also aim to increase expertise in the regional centres on MPS and related diseases in the clinical management of those affected.

The MPS Society supports the regional clinic programme, which can include arranging and co-ordinating dates of the annual programme and funding the tertiary centre doctors' travel and subsistence costs to the regional centres.

Specific arrangements for each clinic and for organising appointments differ for each of the centres. At least one member of the Society's advocacy support team meets individuals and families face to face to offer individual advocacy support.

Birmingham MPS Clinic

24th February 2012
Another bright and early start to attend the Birmingham Clinic. The clinic list is shorter due to being held over two days rather than one.

First to arrive with their dad were Sophie and Nazia, they were both a little quiet but soon showed their talents at table soccer. Natasha came with her family and she slept through all the noise and chaos of the outpatients department.

Mariam looked very snug in her purple coat but decided very quickly it was too hot to keep it on.

Jake had a very cool t-shirt with a meerkat that had the same matching hair colour, although I think Jake needed a bit more hair gel to be a true likeness.

Ali had fun climbing and crawling through the giant tunnel in the play area and was very happy that he didn't have to go back to school after his appointment.

Soon it was time to make my way back home. My thanks to all the team at Birmingham for making my visit an enjoyable one. See you all next time.
Rebecca Brandon r.brandon@mpssociety.co.uk

Photos this page clockwise from top left: Ali Anwar Khan (MPS IV), Jake Glover (Fucosidosis), Natasha Pace (MPS III) & her sister, Mariam Hussain (MPS IV)

Manchester Bone Marrow Transplant Clinic

Friday 4th May 2012
On 4th May the Willink at Manchester Children's Hospital hosted the BMT clinic for children over 6 years old. As usual Jean and the team were busy in anticipation of the impending arrival of children.

Despite setting off from home incredibly early Jordan arrived with his mum in good spirits telling me of his excitement about going to the big school. Melissa looked very smart in her school uniform, whilst Cody decided she was too shy for a photo. As usual Rachel and Charlie arrived together bringing with them all sorts of mischief, but with a little help I was quick enough to catch Charlie for a photo. It was great to meet Jamie and to hear of his amazing achievement of taking part in and completing a bike ride, and I enjoyed a good chat with Steven.

Unfortunately I did have to leave this clinic early due to another engagement so apologies to those that I missed. We would like to thank the team at the Willink for their continued support and look forward to the next one.
Steve Cotterell steve.cotterell@mpssociety.co.uk

Photos clockwise from top left: Jordan Mount, Jamie Topkul, Steven O'Reilly, Charlie Escalonilla, Rachel Rothwell, Melissa Mckie

Birmingham Children's Fabry Clinic

Friday 25th May 2012
It was a lovely sunny, but early start to get to Birmingham. I felt a little weary after being in London at the Palace of Westminster for the MPS 30th Anniversary the previous day.

The clinic started, but didn't… due to the fire alarm going off for the second time that morning. While the alarm is going off no one is allowed to move around the hospital so we had patients outside waiting to come in and doctors in the hospital trying to get to the clinic area! Eventually we got going…

First to arrive and full steam ahead was Cameron and his little brother Connor, trying to get them in one place and to stand still was an achievement.

Double trouble came in the disguise of twins, Keandra and Saskia, specimen pots come to mind when I think of them, but that is another story. Older sister Adriana didn't want a picture taken, well she is a teenager.

Elsie and Freddie arrived with mum and grandma and had fun playing, Freddie decided to adopt another child's grandad, luckily they didn't take him home.

As usual the time goes quickly at the clinic. Thank you to all the team for always making us feel so welcome.Till next time…
Rebecca Brandon r.brandon@mpssociety.co.uk

Photos this page, left to right: Cameron & Connor Merrick, Keandra & Saskia Excell, Elsie & Freddie Brown

The Advocacy Support Service

A week in the life of an Advocacy Officer

Advocacy Officer Steve Cotterell shares his week with us...

Day 1 - After most of the morning in the office catching up with events that occurred over the weekend and preparing for the afternoon meeting I set off with my colleague Rebecca to Kent, the Garden of England. Well, we mainly saw the M25 but at least we didn't get caught in traffic. The agenda of the meeting was to consider the package of care and funding application for Lisa, a lady 40 years old, with MPS III. Lisa's parents contacted us following concerns about how their daughter's care package was to be paid for. Discussions had already taken place and this meeting was to talk through Lisa's needs and to weigh up if it was appropriate to apply for Continuing Healthcare funding, switching from the current social services funds. We were there to support the family through the process and to provide information ensuring that all considerations were taken into account in the assessment.

Day 2 - This day held a new learning experience. I had been asked to meet a family who were fostering a child, 3 years old with MPS VI. The purpose of the meeting was to discuss the condition and go through the different types of support we offer. The family were in the process of applying for special guardianship of the child (something new to me) and so requested our support by providing information to the Local Authority requesting consideration of need both now and for the future. The family have since attended a meeting with the Local Authority and have been awarded special guardianship.

Day 3 - I set off from home bright and early in bright sunshine and headed northwards on the M1 to visit a young lady in Bridlington. I have been supporting this family to access more appropriate accommodation, it had been a struggle, having to file complaints with the director of services, but finally a meeting had been arranged to meet with the Occupational Therapists involved at a new property to discuss the options available.

What I was not expecting was to be scuppered by snow! The further north I went the worse the weather until in the end I deemed it unsafe to continue. I stopped at services to call the family, who were thankfully very understanding, then, with some difficulty and a wheel spin I turned the car around and headed back to the office rather frustrated.

Day 4 - Heading north again, this time no snow, thankfully. I was heading to Lincolnshire for a meeting with the Watsons. Zara, a young lady with MPS III, had spent some time in hospital and we were meeting with the social worker to formulate a plan for discharge home. I have been working closely with the family and the Local Authority to try to establish a suitable package of care, some progress was made but there is still work to be done.

Day 5 - A bank holiday, time for a rest... although my little girl has other ideas!

If you would like support from the MPS Advocacy Team please phone **0845 389 9901** or email **advocacy@mpssociety.co.uk**

All Ireland Advocacy Support

The All Ireland Advocacy Support Service is continuing to grow. Alison Wilson, All Ireland MPS Advocacy Support Officer is delighted to have been able to support many more families since she last updated you on this service. As usual she brings just a brief update on the work we do in Ireland and what we hope to do in the future.

All Ireland Advocacy and Support

This has been a busy season for the All Ireland Advocacy Support Service. Alongside all the usual advocacy work and clinics, we have also had the Irish MPS Society Family Weekend in Kilcuan and All Ireland MPS and Fabry Conference in the Hilton Hotel in Templepatrick. It has been a busy but exciting few months!

This time I'll bring a shorter than usual update on the advocacy work so that I can spend a little more time letting you know exactly what goes on at the Irish MPS Society's Family Weekend and at an MPS/ Fabry Conference – I hope that by finding out more you will be enticed to come along next time. Those testimonials that I promised in my last update will have to wait until next time!

Northern Ireland MPS Clinic

(11th May 2012)

This year the MPS Summer clinic was held on the Friday before the All Ireland MPS and Fabry Conference so there was a buzz of anticipation in the air. Again we had a busy day, with 14 of our members being seen at the clinic. As usual the clinical team (Dr Fiona Stewart, Dr Simon Jones, Ms Aoife Bradley) saw each of the families while I spent time in the waiting area having a coffee and chatting about any support needs they might have. As usual, families brought with them a range of support needs and I have spent the weeks following the clinic liaising with families and supporting them in any way that I can.

Many thanks to the clinical team and to the staff at the Antrim Area hospital for facilitating this clinic.

Fabry Clinic

The last Joint Cardiac and Genetics Fabry Clinic was held on 20th April. This is always a busy clinic. Those attending the clinic arrive in the morning to have their cardiac investigations carried out so that the results are available for the clinic in the afternoon. The time in between the investigations and the clinic consultation creates the perfect window for our members to spend time talking over any support needs they might have. Many thanks to Dr Fiona Stewart, Dr Pascal McKeown, Joanne McOsker (Speicalist Nurse) and Tracy Jardine (Specialist Nurse) for their role in this clinic.

Southern Ireland

During this last season I have been travelling to all the four corners of Ireland supporting families in their homes, in schools and even in council offices! I'm always delighted to hear a new voice at the end of the phone, so please do spread that word and let other MPS families in Ireland know that there is a service to support them should they need it.

Traveller Awareness of MPS

In my last update I let you know that I would be holding the first of a series of Traveller Education Sessions. On the 12th February (on an uncharacteristically lovely day!) I made my way to the Donegal Traveller Project to meet with a team of family workers for the Traveller Community. We had an informal education session where I screened the Hurler Awareness DVD and presented a 'crash course' in MPS, genetic counselling and Bone Marrow Transplant. I was delighted with the response and was inundated with questions from the group about how they can best support families who might be at risk of having a child affected by MPS.

In the coming months I hope to hold more of these sessions at Traveller Groups across Ireland.

MPS Family Weekend in Kilcuan

On 5th May I travelled to Kilcuan (Clarinbridge) to join with the Irish MPS Society and their members at their Family Weekend. Unfortunately I was unable to stay for the weekend (there's always next year!) but I had the pleasure of spending a lovely afternoon with the families who attended. We spent lots of time chatting and getting to know each other before going outside to remember those who are no longer with us by releasing balloons into the sunny Clarinbridge skies.

The Irish MPS Society have used Kilcuan for their family weekends for some time now, and it wasn't difficult to work out why. Kilcuan, is a sprawling single storey bungalow that was designed to meet the needs of wheelchair users and those with special needs. The communal areas of the bungalow are big and airy (with plenty of room for an impromptu game of football!) and the bedrooms are all wheelchair adapted. They even have changing and hoisting facilities on site!

During the weekend families took advantage of the swimming facilities on site, went bowling, visited the local cinema, had lots of laughs and ate plenty of food! I would encourage any families who haven't visited Kilcuan with the Irish MPS Society before to get their names down early for next year – I'm looking forward to it already!

Thanks to the Irish MPS Society for inviting me to be a part of their weekend.

Ready to go our for the day - families and volunteers

Advocacy Support

We are here to help…

FABRY AND MPS CONDITIONS Employment & Support Allowance & Disability Living Allowance

As you may all be aware by bitter experience, it is getting more difficult to obtain Disability Living Allowance and Employment & Support Allowance.

Over the past few months there have been more members contacting us to advise that the DLA has been turned down, and members are also being asked to attend medicals to assess them for the Employment and Support Allowance.

The MPS Society has won appeals and tribunals in respect of the Employment and Support Allowance. To avoid having to go through the stress of appeals and potentially further we suggest that you contact the Society to assist in completing the medical form in respect of the Employment and Support Allowance. This may help to alleviate the need for a medical assessment and subsequent appeal. It appears that every Fabry medical assessment the member has been found to be fit for work and the benefit is then stopped, this then means a period of time elapses to appeal etc., causing potential hardship.

This is the same for the DLA. All the forms can be completed online and we work closely with members to ensure that we have all the relevant issues which affect their health.

So don't struggle…
JUST ASK AND WE CAN HELP!

The Advocacy Support Team
advocacy@mpssociety.co.uk
Tel: 0845 389 9901

Ireland MPS and Fabry Conference

On 11th – 13th May the MPS Society descended on Templepatrick for the Ireland MPS and Fabry Conference, with families and professionals travelling from across Ireland and the UK to be a part of it!

On the first evening of the conference the MPS Society Team, families and professionals had a relaxed evening meal and the opportunity to catch up with old friends (and meet a few new ones!). This is such an important part of any MPS/ Fabry conference and I know that those families who attended the conference really benefitted from talking about their experiences with the only people who really understand – other MPS families. Saturday morning at 9.30am saw the 'kick-off' of both the MPS and Fabry conferences and we were delighted to welcome a group of eminent professional speakers from Northern Ireland, mainland UK and beyond to update fellow professionals and our families about MPS and Fabry diagnosis, management, treatment and their hopes for the future. It's always exciting to hear about new developments and what these developments might mean for families in the future. Many thanks to all the professionals who gave up their weekend to share their expertise.

For me, the stars of the show are always the family speakers! In the MPS Conference we were delighted to hear from: Aidan Kearney and Shane Bell who gave us an insight into what it's like being on a clinical trial; Maureen Walker about how she and her family coped with Charlie's diagnosis and his Bone Marrow Transplant and Helen Lever about how she and her family have adapted to Aaryana's diagnosis over the years. In the Fabry Conference we heard from Colin O'Reilly who let us know what it's like to be a Fabry patient in Ireland. A huge thank you to all the family speakers for taking the step to share their experiences for the benefit of others – you truly are an inspirational group.

I thought the conference was fantastic! I was exhausted but inspired at the end of the weekend. But why take my word for it, please read the reports from some families who attended the weekend. We would be delighted to welcome you to our next All Ireland Conference!
Alison Wilson a.wilson@mpssociety.co.uk

MPS Annual Review and Accounts

The MPS Annual Review and Accounts are now available to download from our website free of charge. Hard copies are also available in print at a cost of £3 to cover printing and postage. Please contact us on **0845 389 9901** or email **accounts@mpssociety.co.uk**

Kevin and Bernie Drayne
(Parents of Roma – MPS IV)
"The conference was great, meeting old friends and new. Every conference brings new information and new issues for us all to focus on. As we all know MPS brings with it a range of changing needs and new solutions. The local aspect was particularly important as we had the opportunity to find out what services are available close to us, and we had opportunities to compare provision between different areas.

I know it is very difficult for 'new' families to attend, but in my MPS journey our first conference - although harrowing - was at the same time a turning point for us, when we met other families living with the same condition who welcomed us with open arms.

Each conference brings hope to us all, and this was particularly illustrated by Aidan and Shane (MPS IV) who were able to tell us about how their lives have changed as a result of being a part of the new and exciting clinical trial for their condition. Apart from all the information we received, we really enjoyed the craic - yes conferences are good fun too!"

Tony and Lorraine Woods
(parents of Rebecca – MPS IIIB)
"We recently attended the Ireland MPS/Fabry conference in Templepatrick (Belfast) and many of the talks were of great interest to us.

Our Rebecca (MPS IIIB) is 19 years old now and sometimes you wonder if you will hear anything new (we have attended so many conferences) - but we did! The talks about research give so much hope. Hearing about the current trials for Morquio Disease and hearing the patients talking about their treatment was fantastic.

It's great to be up close to the best doctors in the world and able to ask questions casually.

The conference also is a great social gathering too, meeting new families and catching up with our old friends. The conference being so close to home cuts out too much travelling – it's difficult now with our Rebecca being less able. Many thanks to our Irish Society for funding assistance, and to the UK MPS Society who organised the Conference. A very worthwhile experience."

"Congratulations on organising an excellent meeting both from a content and social point of view. Please convey my thanks to your colleagues at MPS."
Bryan Winchester

Comments on Facebook
"Thank-you to everyone at MPS for the great weekend conference, plenty of information and plenty of fun, most enjoyable x" Bernie Drayne

"A huge thank you to the whole MPS Team who arranged the Northern Ireland MPS Conference at Templepatrick. It was great. Thanks to everyone who gave up their weekends and put their own families on hold, to be there for us. We really appreciate everything you do."
The Lever family, Warrenpoint.
(MPS I H/S)

Find us on Facebook

Lancashire Family Day

On Saturday 14th April 2012 families arrived at Camelot Theme Park ready for a day of fun together at our Lancashire Family Day.

On arrival the families met two of our Advocacy Team who were there to support them.

Families then had plenty of time to explore the park and the many rides and attractions, including daredevil rollercoasters and high-speed spinning rides for those who were brave enough! There were also lots of gentler rides for the younger ones and those with disabilities, as well as an indoor play area and amusements for when the weather got too chilly! Some families enjoyed a round on the crazy golf course. There was also a Birds of Prey centre and an animal enclosure with the chance to see some cheeky meerkats along with a range of farm animals. Many of the children took the opportunity to have their face painted – it was lovely to see the array of animals, butterflies and other fun designs on their faces.

At 12.30 everyone gathered together in the big arena for the jousting tournament, which provided an opportunity for the families to meet each other and spend more time getting to know each other and our staff.

The show started with a jester fooling around and doing fire-juggling, before the stars of the show arrived – King Arthur and his knights. The families enjoyed the spectacle of the good knights competing against the bad knights in a jousting tournament, as well as some more fun with the jester and pals. At the end of the show there was even a chance to meet the beautiful horses.

After this everyone enjoyed lunch, and then had more time on the rides. Some families watched the Birds of Prey show later in the afternoon, while others relaxed with a hot drink in one of the cafes to keep the chill out. The worn-out families then headed home having had a super day, and having met with others to keep in touch with in the future.

All the families had an amazing time at both events and gave a lot of positive feedback about the much-needed delight these events had brought to their children, both those with MPS and related diseases, and their siblings.

Elizabeth, mother of Jack who suffers from MPS II, Hunter disease, sent us the following comments about the Camelot fun day. (Sadly Jack was too ill to attend, but the family found it so helpful to spend time with their daughter Katie, who, as a sibling, is also in need of such chances to enjoy herself away):

"Even though Jack was too poorly to join us it was lovely to be included and see Sophie [Senior Advocacy Officer] and get the opportunity to catch up face to face. Katie, Jason and I had a wonderful day - the weather could have been kinder but we are used to that living in Lancashire - it was a chance for us to do something different together as a family rather than just one of us being able to take Katie out to just "fly around Tesco" - which we are sometimes lucky enough to do in a week - a nice day out for Katie is worth its weight in gold. Thank you for making us make the effort to attend something official - we don't have enough spare respite to do many things together - though we did go to Tesco on the way home - the three of us - a first for this year too!

The fact that you get us together with other families for the conferences and fun days lets us know that we are not alone and we also get to help each other out as it is amazing what mines of information we become and specialists in our children's problems and where to source things from and how to get help for them - when we get together we can pass this on - even when only saying hello we can sometimes say something which will be of help to another family."

These trips were such great experiences for these families who are so much in need of some fun and friendship. They will be long-remembered by all the families who went, and we are so grateful to the William Brake Charitable Trust for having made this possible.
Sue Cotterell
s.cotterell@mpssociety.co.uk

30th Anniversary Reception at the Palace of Westminster

On Thursday 24th May 2012 the MPS Society, along with Jeremy Hunt MP, hosted its 30th Anniversary reception at the Palace of Westminster. It was a beautiful day and the Terrace Pavilion was the perfect location to hold the celebrations. A selection of both families and professionals joined our celebrations, some of whom kindly spoke of their experiences with MPS diseases but the event was the perfect opportunity for all to reflect on the past 30 years and the challenges overcome not least by the Society itself but by its members, supporters and those who work tirelessly to find a cure to MPS and related diseases.

We would firstly like to take this opportunity to thank all of our speakers: Bob Stevens and James Hope-Gill, who both offered a heartfelt personal perspective into MPS and the effect both the disease and the Society have had on their lives; Dr Fiona Stewart, Consultant in Genetics, Belfast City Hospital, gave a thoroughly enjoyable and insightful speech on the relationship between the Society and clinical teams and the support offered to each other; and Professor Bryan Winchester and Brian Bigger spoke of the incredible research achievements gained in conjunction with medical experts and the opportunities for future developments. And, of course, our Chair of Trustees, Sue Peach who introduced all of the speakers and spoke herself on behalf of the Society.

We would also like to thank Helen Skidmore and Paul Franklin, who both recently celebrated their 30th birthdays, for cutting the cakes. We were delighted to be able join all three landmark birthdays together and raise our glass to many more!

Bringing the celebrations to a close was our host, Jeremy Hunt MP, who offered a heartfelt speech and celebrated all the efforts of the Society together with the support of families and professionals so that we may continue to offer the much needed support to our members.

Finally, thank you to all who were able to join us in making our 30th anniversary special and thank you to all who have supported us, without your support we wouldn't be the Society we are today, thank you. **Joanne Lawley**

Wicked Genes

Creating a Future with Hope for Children with Genetic Diseases

We are proud to launch a new and exciting fundraising initiative for the MPS Society, focusing on increasing opportunities for groundbreaking research and vital advocacy to continue.

Wicked Genes aims to appeal to a wide range of people. We are promoting the initiative across the UK to schools, universities and businesses as a new exciting and fun way to get groups of people connected to raise money for a very very worthwhile cause.

Wicked Genes is about having wicked fun, doing something wickedly crazy, or wickedly daring, or wickedly silly, or wickedly lovely. It's about coming together with friends, workmates and strangers to raise a bit of money to save children's lives.

Wicked Genes needs you to get involved today! We need:

- individuals and families to tell their stories - on the website and to local schools and businesses
- You to tell all your friends and families about this exciting new fundraising opportunity
- to hear about how you are fundraising with Wicked Genes!
- You to visit our website to get more information and download a fundraising pack

There are a number of ways to get in touch:

Give us a call on **0845 389 9901**
send us an email: **wickedgenes@mpssociety.co.uk**
Visit our new website at **www.wickedgenes.co.uk**

We look forward to hearing from you!

MPS Awareness Day

Each year the Society celebrates International MPS Awareness Day on 15 May. This is a day devoted to raising awareness of MPS and Related Diseases. For this year's campaign around Tuesday 15 May 2012, many of you held fundraising and awareness events. Here is a round up of some of your stories...

Ethan's Fight Against MPS, www.justgiving.com/ethanmps

Katrina Fanneran Burley's son, Ethan is 3 years old. Last July he was diagnosed with MPS VI, Maroteaux-Lamy Syndrome.

To celebrate MPS Awareness Day 2012 and help support the MPS Society, Katrina set up a justgiving.com page encouraging donations from people wearing blue on MPS Awareness Day. The page received an overwhelming response raising just over £6100 for the MPS Society.

Katrina's story taken from her Justgiving page reads: "Ethan is an amazing, strong and beautiful little boy. He is full of life, love and laughter and my fight is to keep him that way for as long as possible.

He is under the lifetime care of Great Ormond Street Hospital and undergoes a weekly treatment of Enzyme Replacement Therapy by intravenous infusion which we hope will at least slow down the progression of this disease.

He already has damage to his liver, heart, spine, skeleton, neck, airway, eyes and ears as well as having multiple hernias.

He has already had one surgery and is due to have more, all of which should improve his day to day quality of life.

In all of this Ethan stands defiant. He is unaware of his condition but endures weekly and sometimes daily therapies like they are a piece of cake. He gets upset and frightened of course but a little encouragement from Mummy and lots of hugs and kisses and he soon comes round. He is the bravest little boy and inspires me every moment of every day. He gives me something to live and fight for and he is my little soldier.

So how can you help Ethan? Simple. WEAR BLUE! I want to get as many people as possible to wear blue, be it clothes, shoes, eyeshadow or simply a blue ribbon on May 15th, MPS Awareness Day so as to raise the profile for all MPS diseases and sufferers. My hope is that all who can, will donate to the MPS Society, through Ethan's page which will help other families like Ethan and I and more importantly it will help fund research in the hope of finding a cure."

Awareness Day Coffee Morning

Teresa and Shane, parents of Corey (MPS I) held a coffee morning on Saturday 12 May at Preston Baptist Church Hall to raise awareness and funds for the MPS Society around MPS Awareness Day.

The coffee morning was a huge success which we think over 100 people attended. The weather was beautiful. There was a bouncy castle in the garden, teas, coffees, homemade cakes and a couple of gift stalls. The NHS blood donation service signed up 11 new donors and handed out lots of information to potential blood and bone marrow donors. Our local fire service came along too and children, and many of their dads, got to sit in a fire engine! Most importantly all of these people became aware of what MPS is and got to meet Corey.

It was hard work but a pleasure and I am so glad that we raised £615 for the MPS Society to help you continue the hard work you do to support families like us. **Teresa Jeffery**

Taiwan MPS Society

This year for MPS Awareness Day, the Taiwan MPS Society decided to celebrate it jointly with Mother's Day and awarded nine spectacular mothers with trophies to make this day special. **Virginia Tsai** Taiwan MPS Society

Rooster's Nursery Fundraiser

I just wanted to let you know that Emily Bradshaw's Nursery, Rooster's, did a fundraiser last Wednesday for MPS Awareness Day (albeit a week late). All of the children had to wear blue and bring £2 – they managed to raise £67.

Emily currently goes to Rooster's every Tuesday and Wednesday – it is a lovely little nursery just outside of the village of Betley, Nr Crewe, Cheshire. They are all aware of Emily's condition and wanted to do something to help support the charity. **Gayle Bradshaw**

Elizabeth Heath, mother of Jack MPS II, held an MPS Awareness Day bun sale at her work and raised £60.94. Thank you!

Welly Waddle raises £530

Here are a couple of the pictures from the Welly Waddle we did at Laugh and Learn Preschool to celebrate MPS awareness day on Tuesday 15th May to raise awareness and funds for you as you have helped the family of a little boy who attends our Preschool so much.

William Sainsbury (right) was diagnosed with MPS I in January 2010 and has recently undergone a successful bone marrow transplant at Great Ormond Street Hospital. He is due to start coming to Preschool again at the beginning of June and we can't wait to have him back. We chose a welly waddle as this is William's favourite type of footwear by far! Each child was asked to obtain sponsorship in return for participating in a number of fun activites such as icing a cake, an obstacle course, Welly painting and hunting for hidden objects in jelly! We all had a fantasic time and William and his parents were able to attend too. We raised an amazing £530 from our very supportive and generous parents who also brought MPS ribbons, trolley tags and wristbands. **Sharon Bennett,** Manager at Laugh and Learn Preschool Worthing.

Allens Croft Children's Centre

Lisa O'Connor from Allens Croft Children Centre shared her MPS Awareness Day celebrations with us…

"Just thought I'd let you know about our fundraising day that we held at nursery on 15th May (MPS Awareness Day). We called it '*Bring your wheels to nursery*'. All the children bought in a £1 donation and their bikes, scooters, prams and pushchairs and along with our local community support officers also on their bikes we all went out and rode them around the playground. We made some displays to go up around nursery to highlight the MPS Society and give people a wider understanding of the MPS conditions.

Obviously your charity is etched on our hearts as our Head Teacher Laura Brodie's son Will also lives with this and he is on your 'lend a hand' poster which we love.

Anyway, as a result of our day we have raised £116.45. Your charity is part of me now and I love doing what I can to support all your hard work. A few years ago I created 'Walk for Will' and raised just over £3000. I have been looking at the recent MPS Magazine and noticed the parachute jump and I think this could be the next challenge ahead (even though I'm scared of heights!). **Lisa O'Connor**

MPS Awareness on the buses!

Our son Harry has (MPS I) Hurler. He has had a BMT and his brother Oliver was his donor. We are Lucy and Ian Evans his parents. We are very lucky to have a close family who always listen and help especially Pat, Don, Sylvia, Barry and of course Marg, Lucy's sister. In my work as a bus driver for Arriva the Shires and Essex I am extremely lucky to have a boss who is always willing to help with any problems or time off that is needed for the care of Harry or hospital attendance. **This is our story of MPS Awareness Day 2012…**

We were sat at home just talking one evening and the subject of the MPS Society came up. We both said it was coming round to MPS Awareness Day. I said wouldn't it be fantastic if we did something to raise money and at the same time raise awareness. But what do we do? For a few days we went round with ideas in our minds but nothing concrete. Then I went in to work to see what my boss says about doing things in my workplace. That was the push that started the ball rolling, within minutes he was sending emails to people and suggesting things that we could do. He agreed to a non uniform day with everyone donating and quizzes to try to raise more money. There was even an agreement to having donation boxes on the buses for the public to donate.

I found a suitable quiz for the bus drivers (I decided chocolate bars as all bus drivers eat chocolate), then decided I would give prizes as a thank you to all who were to take part. The main prize being a meal for two at the Hampden Arms in Great Hampden donated by landlords Constantine and Louise Lucas who are regular supporters of the MPS Society. I then wrote to all members of staff saying what was planned. I had one gentleman ask to see me, and he asked would it be ok if he spent the day dressed as Elvis Presley. Of course was my reply. Then I set about the task of printing all the papers I thought I would need. A few days before the 15th we were due to attend Great Ormond Street for an outpatient appointment so I arranged with Antonia to collect a fundraising pack, balloons, leaflets and the donation boxes, from the offices on the way to London. But because of all the rules and regulations that there are with the collecting of monies from the public I had to get emails sent to say we were not asking the public for money. So I just had to go back to Amersham on the Monday and pick them up. Which was not a problem as I had a late start to my day's work (15.45).

The only problem with the late start was the late finish (23.48) which meant very little sleep if I was to be at work for the first person to start at 4.45am. I did make it. I set up a table with leaflets and my quiz sheets on, I also had a glass jar filled with pasta shapes for everyone to guess how many were inside (counted by Lucy) and a chocolate cake where everyone was invited to guess the weight. Then I just had to wait and see how many people would take part. I was astounded by the support and willingness to hear me speak about our experiences with Harry and the MPS Society. Just after seven o clock Elvis entered the building to do his school run. I think it was the longest school run ever with all the children wanting their picture taken with him when they got to school.

After that Elvis spent the day riding round on the buses handing out leaflets and posing for pictures with the public, everytime he left the Bus Station there was a shout of Elvis has left the building. I must admit to having a short break to see Oliver and Harry during the day but stayed at work until half past eight when the last donation box came back. From the events at work I did go straight to the Hampden Arms in Great Hampden where there was a quiz in aid of the MPS Society, and I must say the pub was once again full to the brim with people supporting the Society.

The next day came the moment of truth. We had raised more than the £200 that we had hoped for. I went to see Dennis Mauger who had spent the previous day dressed as Elvis and unbelievably he had raised a massive £403. With the other donations from all the members of staff at Arriva Aylesbury we managed to raise a wonderful £1110. I would just like to thank Steve MacDonald, Steve Ellam, Dennis Mauger and all members of staff at Arriva Aylesbury.

Also a big thank you to everyone at the MPS Society without people like you, people like us would be going through the unknown with no help or advice.
Ian Evans

Through their knowledge of Harry Evans and his family, the Hampden Arms have been wonderful supporters of the MPS Society. They held an MPS Awareness Day quiz and raised £205.00. Louise Lucas from the Hampden Arms writes: "Once again great support from our lovely village with the pub full of friends and family. Boys from a local cricket club won and kindly put their prize money into the pot."

Visit **www.mpssociety.co.uk** for more information
or give us a call on **0845 389 9901** to find out how you can support us…

“We’ll meet again”… in ten years’ time!

Fiona Bingham shares her story of a long friendship that developed as an MPS Childcare Volunteer ten years ago…

In 2002 I temporarily joined the MPS team to support the 7th International Symposium in Paris, and have wonderful memories of my time with MPS both as a support worker and a volunteer carer. I remember MPS had lined up some exciting activities for children – a theme park, the zoo, a River Seine trip and picnic at the Eiffel Tower – and my role was to accompany 11 year old Suet-Li (Rachel), a Morquio sufferer, from Malaysia. I was amazed how far Rachel and her mother Sharon had travelled to attend, as had many others. Rachel was great company and we struck up a lasting friendship as you will read below. At the time I wrote a piece for the MPS magazine which finished off “I know we’ll meet again!”, and I was right!

Time passed and after many years of Christmas cards and occasional emails I was thrilled to hear Rachel, now 21, was coming to do her third year of a law degree at University of Hertfordshire and would be living on the Hatfield campus some 45 minutes’ drive from my home in Chesham.

Let me tell you I have the greatest admiration for Rachel and the way she dealt with the many challenges involved in settling into her new way of life, not to mention getting on with her studies and achieving her law degree. Her newly acquired “Lamborghini” (battery operated wheelchair) was her greatest friend and gave her independence to travel; by bus to her lectures in St Albans, and more excitingly to the nearby Galeria shopping mall. Rachel’s accommodation was in a ground floor flat shared with seven others who included two Malaysian colleagues from Kuala Lumpur on the same course. This trio included a Muslim, a Hindu, and a Buddhist which made meal planning interesting!

During the academic year I visited Rachel every three or four weeks for coffee, a walk round the campus, or a meet up in St Albans. She came to stay with us for Christmas and loved all the hullabaloo of a British family celebration. Mind you, I learnt that although Rachel and her family is Buddhist, they like to make the most of other religious celebrations and always have a decorated Christmas tree!

When I asked Rachel what she hoped to do once she had finished her degree she surprised me by saying she hoped to become a barrister – never one for the easy option. This set me thinking what little thing I could do to help and I began nursing an idea of a day in London, visiting the Inns of Court, and perhaps going into a court case. I asked, explaining the situation, a barrister friend for some advice to which he responded he would be happy to take Rachel for a three day mini-pupillage in his chambers! Wow! I just couldn’t wait to tell Rachel and when I did she was as excited as me and we started making plans.

The dates were set for early June and I was to accompany Rachel for a four night stay in London (…… Paris, London, what international people we were!). By this time Rachel had had some experience of visiting London and was confident to travel by train into London and around town by bus. She was confident but I was getting a bit nervous about it all! With an eye on cost I had booked us into a Youth Hostel a bus ride from the West End to the chambers in Middle Temple, but not long before our trip Rachel’s family booked us into the Strand Palace Hotel at their expense!… Things were looking up!

www.wickedgenes.co.uk

At last Rachel's exams were behind her, she had a place lined up at the Manchester Bar School, and we were off on our adventure, and what a GREAT time we had. The sun shone the whole time, London was at its best, and every single person we encountered was kind and friendly. We arrived the afternoon before the placement was to start in order to locate the right address and find our feet. So, it was a slightly nervous but excited Rachel who next morning launched herself into the rush hour crowds weaving her way in and out for the 15 minute walk to Middle Temple. I thought how proud and happy her parents would be to see her independently striking out about 30 yards in front of me in the Lamborghini on her way to work. My thoughts were accompanied by the 9am bells of St Clement Danes church ringing out "Oranges and Lemons" – I felt in love with London putting on its best show for my friend!

We couldn't have written the script for our trip better. On the legal side Rachel spent half her time independently in the barrister chambers – looking and discussing cases, reading briefs, meeting barristers, breathing in the atmosphere and surveying the scene. The rest of the time I accompanied her to an important tribunal and into the High Court. We had an exciting walk at high speed down Fleet Street accompanied by two barristers discussing and explaining the case with Rachel. At the beginning and end of each day Rachel and I enjoyed a few minutes sitting in dappled shade in the gloriously atmospheric garden court yard watching London's legal people come and go and musing on what a good time we were having.

Out of office hours we were out to enjoy ourselves and we did! We saw the show "Legally Blonde" (how appropriate!) which was on across the road from our hotel. We saw the Thames, Covent Garden and its street performers, Somerset House, Trafalgar Square, Lincolns Inn fields. Rachel has an uncle, aunt, and four cousins who live in London. Rachel and her family are all Chinese so what an experience it was for me to have a meal with them in Chinatown, ordered in Mandarin and all eaten in the Chinese way. After that meal, on a balmy London evening, we all set off down Whitehall, past Downing Street, to see the illuminated Houses of Parliament and Westminster Abbey.

As we parted from Uncle Seven and family we arranged to meet up the next day to see Changing the Guard at Buckingham Palace before Rachel and I set off back to St Albans from St Pancras. You might think Seven is an unusual name for an Uncle but let me explain. Rachel's uncle is the seventh of eleven brothers and one sister and he is the only one living outside Malaysia! His Chinese name is too difficult to say – hence Uncle Seven! As our heads hit the pillow for our last night in London, little did we realise more excitement was in store for us next day. Changing the Guard at Buckingham Palace was off, but instead we had barrier positions for the rehearsal of Trooping the Colour, complete with Prince William! As I said, we couldn't have had a better time.

On a practical note Rachel found moving around London in a wheelchair fairly straightforward and my anxieties were unfounded. Nearly all the pavements had a wheelchair slope, but it was frustrating at times to have to retrace steps if there wasn't one, and, not all the slopes tapered down well enough for easy access. Room for improvement. Rachel was wisely well behaved and always waited for the green man at any crossings, but we missed his help when there wasn't an automated crossing. Disabled access on the buses worked well, but a couple of times the disabled space was already occupied and we had to wait for the next bus. Our railway trip in and out of London worked well with porters appearing promptly with metal ramps at both ends of our journey.

All good things come to an end. In glorious sunshine we got back to St Albans where we parted after our great four day adventure feeling very grateful to all those who had made it possible and to each other. There was more good news to come though when Rachel's exam results confirmed her law degree. I'm sure all readers join me in wishing her well for the future. **Fiona Bingham**

Volunteering opportunities at MPS

Can you volunteer your time caring for MPS children and their siblings at events run by the MPS Society?
Volunteering is fun and rewarding. It could also help you learn new skills and gain valuable work experience.

We are always looking for new volunteers to help out with events and conferences that we run throughout the year.
The MPS Society relies on volunteers to assist in the care needed for children and young adults affected by MPS and Related Diseases. All of our volunteers undertake training in moving and handling and are fully briefed prior to the event.

Becoming a volunteer: The MPS Society accepts volunteers from the age of 16 years on a trainee basis. We will require you to undergo an enhanced Criminal Records Bureau check as the Society supports children and vulnerable adults.
If you are a new volunteer we also require two references and ask you to attend a compulsory training day at MPS House in Amersham. All new volunteers will be mentored by an experienced volunteer.

Please can you help us? Email: mps@mpssociety.co.uk or phone 0845 389 9901

To Celebrate the Saving of Children's Lives Through Newborn Screening

To raise awareness of the important health benefits for children that the extension of the Newborn Screening programme can bring to the UK and the fact that some children are needlessly dying, the Patient Advocates for Newborn Screening Group (PANS) organised a successful newborn screening awareness event at the Terrace Pavilion, Westminster on 25th April 2012.

At the event, children who had been diagnosed through newborn screening for our current five disorders and their families, met with MP's, Members of the Lords, Health Select Committee members, clinicians, scientists and representatives from the European Union, to celebrate the fact that their lives had already been saved. At the same time as having a great party, children and young adults were able to speak to this cross section of Members of the House and health professionals on how their own lives had benefited by early diagnosis and treatment of their disorder through newborn screening and importantly to raise awareness of how other children's lives could benefit by extending the programme.

The event was opened by the actress Miss Jenny Agutter and Mr Stuart Andrew MP for Pudsey and we thank them for their interest and support. Miss Nicola Bramley who has PKU gave a very moving speech on how immediate treatment allowed her to achieve many significant things in her life which would not have been possible without the detection of her disorder through newborn screening and diagnosis at birth. Mr David Elliman of the UK National Screening Committee attended to say a few words.

Across the United Kingdom we screen babies for only 5 inherited metabolic diseases at birth whilst other countries screen for many more. In 2012 the UK is 10 years behind the USA, (now screening for between 30 and 60 disorders) and 5 years behind Australia and New Zealand, (28 disorders) and some EU countries e.g. Austria (29), Spain (27), Iceland (26).

Newborn Screening in the United Kingdom was first introduced for Phenylketonuria (PKU) in some local areas in the 1950's, however this was introduced nationally in the UK in 1969. PKU was followed by the introduction of screening for Congenital hypothyroidism (CH) in 1980, Sickle cell disorders (SCD) in 2006, and Cystic Fibrosis introduced nationally in 2007. In response to 3 reports from the UK Health Technology Assessment (HTA) research programme in the 1990's and a limited pilot study, screening for Medium-chain acyl -CoA dehydrogenase deficiency (MCADD) was finally introduced from 2009 onwards, over a decade from discussion to implementation. As evidence shows, getting a new disorder accepted onto the UK screening programme is an extremely long process.

The Department of Health has announced a one year pilot from July 2012 to screen babies for a further 5 diseases across parts of the UK. However the pilot only covers 60% of all babies born in the UK. 4 out of every 10 children born, i.e. in some parts of England, in Scotland, Wales and Northern Ireland will not be screened. Whilst the lives of some babies will be saved, almost as many may be disabled or die because the whole of the UK is not being covered. Yet another health postcode lottery but this one putting newborn babies at risk. There is no decision by the DoH on what will happen at the end of the pilot or on how long it will take to roll this out across the whole of the UK. A further 5 disorders still falls well short of the number of disorders that some other countries are screening newborns for.

One of the outcomes from the April Westminster event is that PANS are now working with Mr Stuart Andrew and other selected MP's on developing a further strategy for securing Government and Department of Health commitment to extending the newborn screening programme.

Report on Eurordis Activities

I am very honoured to serve on the Eurordis Drug Information, Transparency and Access Task Force (DITA). DITA met on 3 May 2012 by conference call to receive feedback on the 'consultation on the new pharmacovigilance legislation adapted by the Council of Europe and the European Parliament published on 31 December 2010 and coming into force in July 2012. Eurordis through its representative Francois Houyes highlighted the main points of this legislation particularly the patients' perspective.

The European Agency has published guidelines on pharmacovigilance. One of them is that 'Member states including the United Kingdom, should adopt all appropriate measures to encourage patients' feedback on adverse reactions. http://ec.europa.eu/health/human-use/pharmacovigilance/index_en.htm

Eurordis' role has been to indicate which measures are the most useful to patients including documents, training and helplines and any other useful tools. Possible actions expected by Member States could include:
Support including financial support for helplines that can assist patients and/or their families reporting adverse reactions.
Organisation of training for responders on the tools used to report adverse events.

The new pages to the EudraVigilance website giving public access to a comprehensive analysis of the undesired effects of orphan drugs/treatments/therapies have not yet been published. As soon as this is done MPS will communicate the information via the MPS Magazine and on the MPS website.

The DIOD project: Daily Impact of Orphan Drugs in Patients' Lives is a new project by Eurordis and partners to increase knowledge on the medicines/treatments/therapies patients with rare diseases are taking, both on desired and undesired effects. Post-marketing surveillance is key to monitor orphan medicinal products and also medicines used off-label. The DIOD project will work towards identifying ways to involve large numbers of patients in this surveillance by promoting spontaneous reporting. **Christine Lavery**

Fabry International Network

The Fabry International Network (FIN) carried out a survey between late 2010 and early 2011 to evaluate the largest patient perspective possible through the member Fabry Groups of FIN. This abstract of the methodology and findings was accepted as a poster at the WORLD Conference in San Diego in February 2012.

The Fabry International Network (FIN)
Fabry Treatment Survey
www.fabryintnetwork.com

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Introduction

As a global organization based in the Netherlands the purpose of the FIN Fabry Treatment Survey was to evaluate the largest patient perspective possible concerning the impact of the ongoing Enzyme Replace Therapy (ERT) Fabrazyme® shortage and restricted supply of Replagal®.

Background

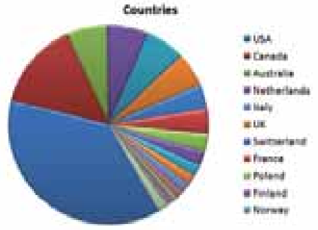
The Fabry Treatment Survey was developed by FIN in collaboration with its Medical Advisory Board (MAB) including input from FIN's three primary sponsors; Amicus Therapeutics, Genzyme/Sanofi and Shire HGT. The survey was conducted in late 2010 – early 2011.

Methods

- FIN designed an online survey in English.
- It was translated into French, Italian, German, Polish, Portuguese, Spanish and Dutch.
- 27 questions: multiple choice / 3 open ended response.
- The survey was sent to FIN members of 24 organisations in 22 countries.
- The survey was open to all Fabry affected men, women and children without regard to treatment.
- FIN collected responses and translated into English.
- Analysis was completed by professional medical research company.

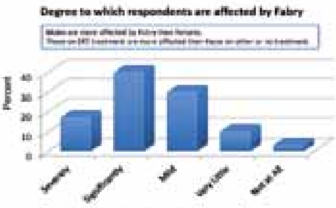
Respondents by Region

- Responses came from North America 52%, Europe 39% and all other regions 9%
- Response locations were dominated by English speaking regions being; US 39%, Canada 14% and Australia 7%.
- Total number of respondents before deadline was 442
- The mean average age of survey participants was 45 years. Ages ranged from 18 to 65.
- Males and females responded in equal numbers,
- The survey was not distributed in all countries receiving ERT and results may not be representative of the Fabry patient community at large.



Degree Respondents Where Impacted by Fabry

- Over half of respondents say they are severely or significantly affected by Fabry disease. Few are not affected. Males are more affected by Fabry than females and those on ERT treatments are more affected than those on other or no treatment.



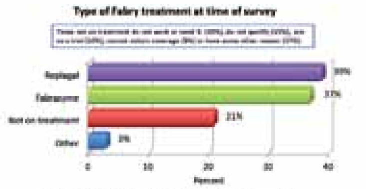
Base: Total answering (n=424)
Q. 4: How would you say you are affected by Fabry disease?

Fabry International
F.I.N.
Network

The Fabry International Network (FIN) is an independent nonprofit organisation representing the global Fabry community comprising of 27 member organisations from 24 countries.

Type of Fabry Treatment at time of survey

- At the time of survey most respondents were on ERT. Those on ERT have been on treatment for a mean of 6 years.



Base: Total answering (n=425 for Q. 5, n=341 for Q. 8)
Q. 5: Which treatment are you currently on for your Fabry disease?
Q. 8: How long have you been on treatment?
Q. 6: If you are not on treatment why?

Impact of Shortage on Health and Well-Being

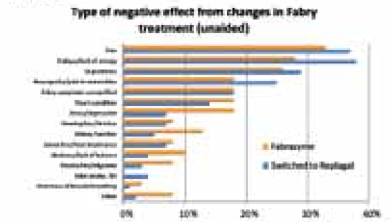
Limitations

- In interpreting the positive and negative effects, please note that the base sizes are not the same. About 140 respondents who had a change in medication said they had some sort of negative impact from the change. Only 42 of these same individuals said they had a positive impact and only 14 said they felt better or had fewer symptoms.

The negative effects since being on reduced dose of Fabrazyme®, transferring to Replagal® or stopping treatment.

- 21% had no negative effects, 24% had no answer and 55% reported negative effects of those whose treatment was affected. Physical symptoms, especially pain, fatigue, GI problems and neuropathy are the most common problems.

- Those on Fabrazyme® more often reported a negative effect (65%) than those who switched to Replagal® (51%). However, those with problems reported similar negative effects.



Base: Responses whose Fabry treatment has changed since supply disruption (n=270). Those with negative side effects (n=94 on Fabrazyme®, n=39 switched to Replagal®).
Q. 24: Since being on reduced dose of Fabrazyme®, transferring to Replagal® or stopping treatment, has there been any negative effects? If so, please discribe.

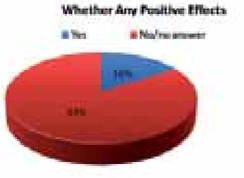
- A majority of those whose treatment was affected report a negative impact. Physical symptoms, especially pain, fatigue, GI problems and neuropathy, are the most common problems.

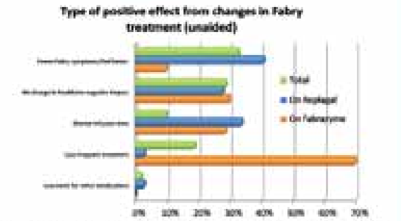
Positive effects for those on reduced dose of Fabrazyme®

- Very few reported a positive impact after changing treatment. Those on a reduced dose of Fabrazyme® like having more free time. Some who switched to Replagal® reported improved health and shorter infusions.

Participant responses are personal comments.

All treatment decisions should be made with a qualified physician.





Base: Responses whose Fabry treatment has changed since supply disruption (n=270). Those reporting positive effects (n=42).
Q. 25: Since being on reduced dose of Fabrazyme®, transferring to Replagal® or stopping treatment, please tell us of positive effects.

Conclusions

- Male patients on ERT were generally more affected by Fabry than females.
- The number of females responding to the survey was equal to the number of males.
- Even though the survey was distributed in seven different languages the predominantly English speaking countries dominated the responses.
- Communication about the supply disruption reached the majority of patients in a reasonable time frame, but improvements could be made.
- Fabry patient organisations did the best job of informing the patient community about the shortage.
- Globally the majority of patients on Fabrazyme® has been on a reduced dose and has missed treatments.
- In the US and some other regions, Replagal® is not commercially available as it is not licensed and therefore unavailable as a form of treatment. The results presented are dominated by responses from the US and Western Europe. Given that response patterns differ greatly by country/region, results should not be considered representative of global experiences.
- The analysis presented is based on a collection of 442 individual responses representing a proportion of the global patient population receiving Fabry treatments, and so has value both as feedback for FIN, Fabry Stakeholders and the Fabry population as a whole. It may also be useful for other groups facing similar difficulties in the future.
- Due to a number of limitation this survey may not be representative of the Fabry patient community as a whole.

FIN thanks all those that responded to the survey providing highly valued insights into personal experiences.

The author(s) declare that they have no competing interests.

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Sanfilippo research

In December 2011, a conference was held in Geneva Switzerland focussing on MPS III Sanfilippo disease.

During the meeting Prof John Hopwood presented the first interventional study on intrathecal enzyme replacement therapy (ERT) in mice with Sanfilippo disease. He presented data that showed if the treatment was started after symptoms present, ERT alone is not expected to fully reverse the damage of the disease. This means that therapy may stop the disease but residual brain and central nervous system damage would not be fully corrected. In a complementary study carried out by Prof Hopwood and Dr Kim Hemsley it was shown that infant, presymptomatic mice would develop almost normally if treated very early.

Professor Gregorz Wegrezyn and Dr Brian Bigger presented data on the use of Genistein. Professor Wegrezyn stated that the synthetic version of genistein is highly superior to the natural version. Assays of the supplement version of the compound showed very wide variation in the concentration of the active ingredient. Dr Wegrezyn also suggested that many of the natural formulations contained compounds that seemed to have an adverse effect on the disease in vitro.

Dr Bigger described in detail experiments in mice using high-dose pure genistein. He showed that several months into the study the untreated Sanfilippo mice were hyperactive and showed a lack of fear, whilst the Sanfilippo mice that were treated with high dose genistein were calmer and displayed a normal sense of fear. Dr Bigger is collaborating with the UK MPS Society to launch a pioneering double-blinded, placebo controlled genistein clinical trial at the University of Manchester.

Dr Patrick Haslett from Shire HGT presented data from the MPS IIIA Natural History Study. Shire has identified several new biomarkers and learned a lot about the disease.

Key findings of the study include:

Brain volume of MPS IIIA patients decrease with time. This decrease has shown to correlate well with cognitive decline, as measured by the ratio of cognitive age to chronological age. Brain volume can be computationally measured by analysing MRI data, a completely non-subject measure.

This is important because it shows that purely objective and analytical tests can accurately predict an important but subjective clinical measure, cognitive ability.

Cerebrospinal fluid GAG levels are fairly constant with time. That means the GAG levels do not increase with disease progression

Two new biochemical markers were found through an assay of biological material sampled as part of the natural history study.

Patients diagnosed before the age of 6 years tend to demonstrate a more rapid rate of decline than those diagnosed aged 7 or older. The data were provided separately from the mutation so there is no way to correlated age of diagnosis from mutations (www.clinicaltrials.gov/ct2/shaw/NCT01)

Taken from article published in the National MPS Society magazine.

Manchester Research Group on Brink of Developing Treatments

A research group at Manchester University is at the point of developing a range of treatments for childhood genetic diseases having established proof of principle for a number of basic mechanistic approaches.

The Manchester based Stem Cell and Neurotherapies group is developing therapies for childhood brain diseases including Hurler and Sanfilippo syndromes. The lab was set up at the Royal Manchester Children's Hospital and University of Manchester by Dr Rob Wynn, Prof Ed Wraith and Dr Brian Bigger. The nine-strong research team is using a bench to bedside approach to study basic disease mechanisms and translate resulting therapies into treatment for kids with these diseases.

The group has recently described two novel treatments for Sanfilippo disease in work published in Public Library of Science One, and Molecular Therapy. Dr Bigger described how "daily use of the drug genistein is able to delay disease onset and progression in mice with Sanfilippo type B, whilst gene therapy to replace the missing enzyme in stem cells can correct disease in mice with Sanfilippo type A.

Professor Wraith explained "Making therapies like these available to patients requires clinical trials where we can see how well treatments work in patients. Sanfilippo has no treatments, so this kind of work is critically important to children and families with this disease."

Dr Wynn commented "Currently we can use bone marrow transplant as a partly effective treatment for Hurler syndrome but this treatment doesn't work at all in Sanfilippo disease. By using gene therapy to deliver more of the missing enzyme in a bone marrow transplant we hope that we can develop this approach in a clinical trial for Sanfilippo in the near future and for other similar diseases later on."

The team is seeking funding for these two clinical trials and further work to continue studying how Hurler and Sanfilippo diseases occur so they can develop more effective treatments in the future.

More information can be found at:
www.medicine.manchester.ac.uk/staff/BrianBigger

Current clinical trials in the UK

MPS II Intrathecal ERT

Shire Human Genetic Therapies is sponsoring a clinical trial at Birmingham Children's Hospital. The phase I/II safety and ascending dose ranging study of idursulfase administration via an intrathecal drug delivery service in paediatric patients with MPS II is designed to learn whether direct administration of recombinant enzyme into the fluid around the brain and spinal fluid is safe and a possible treatment for children cognitively impaired by MPS II. All participants must be receiving treatment with Elaprase prior to participating in this study.

MPS IIIA Intrathecal ERT

Shire Human Genetic Therapies is sponsoring a "Phase I/II Safety/Tolerability, Ascending Dose and Dose Frequency Study of Recombinant Human Heparan N-Sulfatase (rhHNS) Intrathecal Administration Via an Intrathecal Drug Delivery Device in Patients with MPS IIIA". The Phase I/II Clinical trial began in June 2012 and is currently coming to an end. Patients who have completed all study requirements in this clinical trial are being invited to participate in an open-label extension study that is designed to evaluate long-term safety and clinical outcomes of intrathecal administration of rhHNS. www.clinicaltrials.gov.uk

MPS IIIB Observational Prospective Natural History Study

Shire HGT announced in January 2012 that it will sponsor a natural history study for individuals with MPS IIIB. This study is expected to begin recruiting once the required ethics committee approvals are secured. This is expected by the beginning of July. When the study begins recruiting patients, the location of the study sites and details of the inclusion and exclusion criteria will be posted on www.clinicaltrials.gov.uk. The objective of this study is to evaluate the natural unaltered disease progression of MPS IIIB and has been designed similarly to an interventional clinical trial. This is a longitudinal, prospective, observational, natural history study to identify end points that may be used for future ERT trials via standardised clinical, biochemical, neurocognitive, developmental, behavioural and imaging measures. It is expected that the outcome from this natural history study will provide key disease insights, similar to what would be treated in an untreated control group of patients in an interventional clinical trial.

MPS IVA Enzyme Replacement Therapy

In February 2012 BioMarin Pharmaceutical Inc. closed the access gate to its Pivotal Phase III trial for N-acetylgalactosamine b-sulfatase (GALNS or BMN110) intended for the treatment of MPS IVA having recruited over 160 patients in 40 centres of the world. Recruitment is continuing for three further studies:
Non ambulatory individuals with MPS IVA study;
Study of BMN 110 in Pediatric Patients < 5 Years of Age With Mucopolysaccharidosis IVA (Morquio A Syndrome);
Safety and Exercise Study of BMN 110 for Morquio A Syndrome

For further information go to www.clinicaltrials.gov.uk. If you are not already in a MPS IVA study and are interested to know more please discuss with your child's paediatrician or your physician and/or contact the MPS Society.

Other clinical trials:

MPS I Intrathecal ERT, Los Angeles Biomedical Research Institute, Harbor – UCLA, California, USA

MPS I Intrathecal ERT for Children Being Considered for Transplantation
University of Minnesota, Minneapolis,USA

BioMarin Agrees to Acquire Biologics Manufacturing Plant in Ireland from Pfizer

New facility will greatly expand manufacturing capacity and diversify risk

June 23, 2011 – BioMarin Pharmaceutical Inc. announced today that it has entered into a definitive agreement to acquire a bulk biologics manufacturing plant from Pfizer, located in Shanbally, Cork, Ireland. The plant, which was completed and validated in 2009, is built on ten acres occupying 133,000 square feet of floor space. It was approved by the Irish Medicines Board in 2010. The purchase price is $48.5 million, approximately one-fifth of the expected cost to construct and validate a new facility. The purchase is expected to close in the third quarter of 2011 following the wind down of current operations and the transfer of the Irish EPA license.

The plant will be occupied in a phased transition with substantial manufacturing activities being tied to results of the ongoing Phase 3 clinical study for N-acetylgalactosamine 6-sulfatase (GALNS) for the treatment of Mucopolysaccharidosis IVA (MPS IVA, also known as Morquio A Syndrome). In the meantime, maintenance expenses for the facility are expected to be approximately $4 million a year. It is anticipated that the facility will be licensed for GALNS production by 2015.

Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin stated, "The new plant in Shanbally greatly expands our manufacturing capacity to accommodate our growing commercial portfolio and advancing clinical programs. Our recently expanded manufacturing facility in Novato, California is on track to receive approval by the end of 2011 and can support approximately $1.0 billion in revenue. We believe that additional manufacturing capabilities beyond our current resources will be needed to support anticipated peak sales for GALNS, PEG-PAL for PKU, BMN 701 for Pompe disease, BMN 111 for achondroplasia, if they continue to progress to approval, and our other preclinical programs."

Mr. Bienaimé continued, "The new facility in Shanbally also diversifies our manufacturing risk and provides us with an attractive business environment. The facility is state-of-the-art, utilizing disposable technology and flexibly designed and will allow us to run either fed batch or perfusion processes. This gives us tremendous latitude for the types of products that can be produced at the plant and allows us to focus efforts on the technical transfer of our next commercial product into the facility."

Barry O'Leary, Chief Executive of the Irish Investment and Development Agency (IDA Ireland), welcomed the investment, saying, "This is a strategically important development for BioMarin as it is the first time the company has placed internal biopharmaceutical production activities outside of the U.S. This is the next phase in BioMarin's expansion into Ireland's life sciences industry, following the establishment of an international supply chain and logistics presence in Dublin earlier this year. The decision to acquire the Shanbally plant is a significant endorsement of Ireland's wealth of talent and expertise and it is hoped that this investment will create over 100 high quality jobs in the next five years."

Mr. O'Leary continued, "Ireland has a long, successful track record of attracting significant pharmaceutical investment from multinationals. We have now established Ireland as the second largest development and manufacturing location in the world for biopharmaceuticals, after the U.S. I am delighted to welcome BioMarin, a leading global biopharmaceutical company to Ireland and look forward to working closely with the company as it develops its operations here."

BIOMARIN

Shire

One Year Data Provide Evidence of Effectiveness and Tolerability of REPLAGAL® (agalsidase alfa) in Fabry Patients

Data from Switch and Treatment-Naïve Patients Presented at the 2012 American College of Medical Genetics and Genomics Annual Clinical Genetics Meeting

Nyon, Switzerland – March 29, 2012 – Shire plc (LSE: SHP, NASDAQ: SHPGY), the global specialty biopharmaceutical company, presented data that show favorable treatment effect and tolerability of REPLAGAL® (agalsidase alfa) in Fabry patients who switched from Fabrazyme® (agalsidase beta) or were naïve to enzyme replacement therapy (ERT) after one year of treatment with REPLAGAL at the American College of Medical Genetics (ACMG) annual meeting in Charlotte, NC.

Fabry disease is a rare, X-linked, lysosomal storage disorder caused by insufficient activity of the alpha-galactosidase enzyme. As a result of this deficiency, patients experience a spectrum of signs and symptoms including impairment in renal and cardiac functions. The natural course of Fabry disease includes decrease of renal function and increase in left ventricular mass index (LVMI).

"It's common for Fabry patients to suffer from chronic kidney and cardiovascular disease; these are signs of disease progression," said Dr. Ozlem Goker-Alpan, Director at Lysosomal Disorders Research and Treatment Unit, Centre for Clinical Trials, Fairfax, VA. "These interim results show the effectiveness and tolerability of REPLAGAL in Fabry patients after one year of treatment."

Data Suggest Favorable Treatment Effect and Tolerability with REPLAGAL at the Recommended Dose of 0.2 mg/kg Body Weight after One Year of Treatment

One year data from Shire's HGT-REP-059 multicentre, open-label treatment protocol, suggests that cardiac structure, as measured by LVMI, remained stable in treatment-naïve and switch patients after one year of treatment with REPLAGAL. In treatment naïve patients (n=22), mean LVMI was 48.5 g/m2.7 at baseline and 50.7 g/m2.7 at 12 months, an increase of 2.20 g/m2.7 ± 1.63; p=0.187. In switch patients (n=39), mean LVMI was 60.4 g/m2.7 at baseline and remained virtually unchanged at 12 months at 60.3 g/m2.7 (0.00 ± 2.32; p=0.306). The small increases of LVMI observed in the study were not statistically significant and were below the progression expected from natural history data.

The data show no significant decline in renal function as measured by estimated glomerular filtration rate (eGFR) in treatment-naïve patients (n=29); the mean change from baseline, 83.85 ± 7.12 mL/min/1.73m2, to 12 months was -1.29 ± 4.34 mL/min/1.73 m2, p=0.175. Results were similar in switch patients (n=62), the mean change from baseline, 81.96 ± 4.57 mL/min/1.73m2, to 12 months was -3.17 ± 1.69 mL/min/1.73m2, p=0.009.

Biologic activity by reduction of pathological substrate globotriaosylceramide (Gb3) was also demonstrated by decline in both plasma Gb3 (-2.60 ± 0.52 nmol/mL; p<0.001 and -6.70 ± 1.18 nmol/mL; p<0.001 for switch [n=56] and treatment naïve patients [n=25] respectively) and creatinine normalized urine Gb3 (-0.87 ± 0.37 nmol/mg; p=0.009 and -2.11 nmol/mg; p=0.021) for switch and treatment naïve patients respectively.

The data suggest that REPLAGAL was generally well tolerated. Safety and tolerability was consistent with the known safety profile of REPLAGAL and no new safety concerns emerged in naïve or switch patients over one year of treatment. The safety of REPLAGAL has been well established in over 15 years of clinical and commercial experience.

This study was established to provide US Fabry patients with access to REPLAGAL during the Fabrazyme supply shortage. More than 2,800 Fabry patients are being treated with REPLAGAL globally.

Shire withdraws Food and Drug Application for Replagal ® as a Treatment for Fabry disease

On 14 March 2012 Shire plc announced that it has withdrawn its Biologic Licence Application (BLA) for Replagal ® with the Food and Drug Administration (FDA).

Shire has been in ongoing discussions with the FDA since the supply shortage of Fabrazyme, the only US approved treatment for Fabry disease.

In 2009, and again in 2011, the FDA encouraged Shire to submit an application for the approval of Replagal. The information in the application included relevant updates such as manufacturing and open long-term clinical trial data.

These discussions led Shire to file a BLA last November in anticipation of a quick review process and eventual approval. This would have allowed Shire to be reimbursed for its supply of Replagal and supply US patients with a therapy they desperately need at this time.

Recent interactions with the FDA have led Shire to believe that the FDA will require additional controlled trials to achieve the approval of Replagal, a product licenced in Europe for nearly a decade.

No concerns over Replagal's safety profile were raised by the FDA. However, Shire has concluded that the likely additional studies would cause a significant delay, and an approval of Replagal for US patients would only be possible in the distant future. Shire has therefore decided to withdraw its Biologistics Licence Application.

(compiled from Press Release
14 March 2012)

www.wickedgenes.co.uk

Morgan's WONDERLAND

A place where everyone can play

If any UK MPS families are choosing the USA as their holiday destination and your travels take you to Texas, you might want to check out Morgan's Wonderland. Located in San Antonio, Texas, Morgan's Wonderland is the world's first ultra accessible family fun park designed specifically for children and adults with special needs, their family members, carers and friends. This 25 acre park is a unique oasis which, through the spirit of inclusion, allows people of all abilities to play, learn and share life – changing experiences together in a fun and safe environment. For more information go to **www.morganswonderland.com**

The Challenging Behaviour Foundation

The Challenging Behaviour Foundation would like to announce the publication of the Spring 2012 edition of our newsletter, "Challenge". The focus of this edition is on healthcare for people with learning disabilities, a timely subject given the recent publication of Mencap's follow up report to "Death by Indifference". "Death by indifference: 74 and counting – A progress report 5 years on" highlights failings in the NHS, which has resulted in the premature deaths of some of the most vulnerable in society.

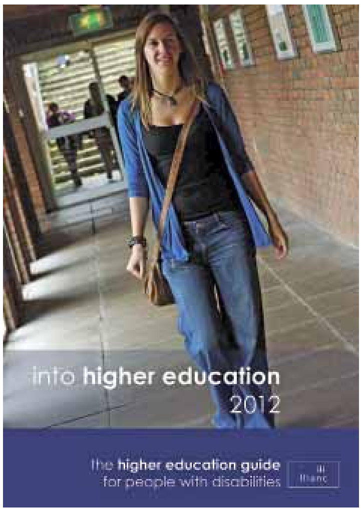
Two family carers share their experiences of the difficulty they and their family member has faced when trying to access necessary medical investigations and treatment, and discuss the barriers they have encountered.

Learning disabilities or challenging behaviour should not be a barrier to receiving good healthcare. The article "Accessing healthcare" written by Darryl Chapman, Acute Liaison Nurse for Vulnerable Patients, looks at the importance of making 'reasonable adjustments', (Equality Act, 2010) and says that accessing healthcare is achievable but it requires good planning, making 'reasonable adjustments' tailored to individual need and for family carers and professionals to communicate effectively and work in partnership.

The CBF celebrates our 15th anniversary this year. This issue looks back at how the charity has developed over the last 15 years, since starting from humble beginnings in Vivien Cooper's garage with a 'borrowed' photocopier and the help of some dedicated friends who worked as volunteers, to having a team of 11 staff and the creation of a national strategy group (the CB-NSG) committed to raising standards for people with learning disabilities whose behaviour challenges services.

The CBF works alongside leading organisations and individuals within the field of learning disability (such as Professor Jim Mansell who sadly lost his battle to cancer in March this year) to raise awareness and improve the quality of life for those who have severe learning disabilities and behaviour described as challenging and their families.

For a copy of the Spring 2012 edition of "Challenge" visit our website: www.challengingbehaviour.org.uk or email your details to: info@thecbf.org.uk to be added to the newsletter mailing list.



Into higher education 2012

A guide to education for disabled people

into higher education 2012, the high education guide for people with disabilities, by Tony Stevens and Barbara Waters (Disability Alliance, 2011) is a free guide for disabled students thinking about studying in higher education.

The guide will help with questions such as whether the college or university will be accessible, how to choose a course and what support will be available. The 2012 edition includes the latest information on tuition fees, repayment methods and student support. You can also read about disabled students' experiences and the challenges they have faced in higher education. The guide also contains a useful resources section listing further websites, publications and organisations which can help.

You can download the guide from the Disability Alliance website:
www.disabilityalliance.org/skillintohe.pdf

Fundraising

Welcome!

The MPS Society supports children and adults affected by MPS and related diseases throughout the UK, their families, carers and professionals. As you will have read earlier in this magazine, we provide a unique needs-led advocacy service, organise and manage events to enable those affected to come together to share experiences and learn about the latest developments in clinical management and treatment, and fund and encourage research into these devastating diseases.

But to do this we really do rely on the generosity of people like you to help us continue our vital work.

We have a whole range of resources available to download from the MPS website including our **Fundraising Pack** which gives you plenty of ideas and guidance on fundraising for MPS plus template forms for you to download, from organising an event, to simply making a donation online or via text.

We can send out T-shirts, balloons, stickers, posters and leaflets to promote your fundraising and we have a range of merchandise to raise awareness of MPS. You can make a difference today.

If you would like to get involved with MPS Fundraising, please email **fundraising@mpssociety.co.uk** for more information…

We would love to hear from you!

This section of the MPS Magazine is devoted to your fundraising stories, opportunities and thank yous! Please read on…

Elvis Tribute Night inspired by Blaise

On 7th April 2012 we held an Elvis Charity Night in Stoke on Trent to help Blaise who has MPS III (Sanfilippo) raise funds for the MPS Society.

It was a lovely evening. Gordon Hendricks, Stars in their Eyes winner 2005 and European Elvis champion 2011 did a fantastic show. Also his son Jamie performed for us and did us proud too.

We raised a total of £2,420 for the MPS Society. Gordon Hendricks so kindly auctioned the leather trousers he wore for the final of Stars in their Eyes and David Walton, the owner of Bidds in Stoke who let us use his venue on the evening, auctioned a replica ring like one that Elvis Presley had worn to help us boost our funds. Thomas Wale had his back waxed ouch! All for our good cause, we also had a raffle which lots of people had donated prizes to.

I would also like to mention that on 28th March 2012 Michelle Fox and her friends did parachute jumps in aid of the MPS Society which was a seperate fundraising event to ours. Fiona and myself would like to thank everyone for the help and support they gave us not only on the evening but leading up to it. Blaise had a wonderful time and would like to thank everyone too for helping to support his charity.

Gordon Hendricks as Elvis has kindly recorded a single 'The Twelfth of Never' and all proceeds from the sale of this single is going to the MPS Society this can be down loaded from I-tunes. A big thank you to everyone who supported us in our fundraising.

Christine Whittle (Blaise's Nan)
& Fiona Green (Blaise's Aunty)

Blaise, his mum, dad, sister Riannon with Gordon and Jamie Hendricks.

Michelle Fox and friends - sponsored skydive for the the MPS Society

Grants make things happen

As the MPS Society improves and expands its services in response to members' needs, grant-making bodies are playing an increasingly important role in providing the funds to make this happen.

We make carefully-targetted applications to suitable trusts and foundations for specific areas of our work and understand the desire of grant-makers to see exactly where their money is going and what it is achieving. This might be to give a group of young carers a respite holiday, provide support to patients and their families at regional MPS clinics or offer bereavement support.

We also welcome contributions towards the core costs of roviding our nationwide advocacy service and are particularly keen to establish long-term relationships with trusts and foundations whose aims and mission are a close match with ours.

If you would like to know more about the difference a grant can make to our work or know of a Trust or Foundation which we could apply to for a grant, please contact us on **0845 389 9901** or email **grantsandtrusts@mpssociety.co.uk**.

Many grant-giving trusts and foundations prefer to give grants for our work in specific regions of the UK. When applying, we look at how many of our members live in these regions and the advocacy support we provide there, and then report back on the work we have done with these families after we have received a grant. Some recent examples of region-specific grants include the Leathersellers' Company Charitable Fund, who have supported our work with families living in Greater London, and the Eveson Trust, Sheldon Trust and Baron Davenport's Charity, who have provided grants for our advocacy service in the West Midlands.

West Midlands Advocacy: The Eveson Trust, The Sheldon Trust, and Baron Davenport's Charity

We have been very pleased in recent years to have received ongoing support from the Eveson Trust towards our services for families in Birmingham and the West Midlands, Herefordshire and Worcestershire, with several much-appreciated grants being awarded to us since 2006. In 2011 we received our first grant from the Sheldon Trust, who also have an interest in the West Midlands region. Last year we were also pleased to receive our first grant from Baron Davenport's Charity for our work focused on Birmingham and the surrounding area. We have around 100 families living in the West Midlands, and our advocacy team has been advocating the rights and needs of these affected individuals and their families in their dealings with health, housing, social care and education authorities, and many related issues.

Examples of support recently provided to West Midlands families: Natasha

Natasha (aged 16) has Sanfilippo disease, and her family has had ongoing issues with housing adaptations. Despite adaptation works being completed at the family home in order to provide Natasha with a downstairs bedroom and bathroom, her parents felt that the bedroom was not appropriate for their daughter and not big enough to manage her needs, and were carrying her up and down a steep staircase to an upstairs bedroom. Due to her recent decline this is no longer possible and is having a negative impact on their health. Our Advocacy Officer Steve contacted the family and arranged to visit them at their home to look at the current provision, and arranged to meet their Occupational Therapist at the same time to reassess the current adaptation. From this meeting it was agreed that the provision was not entirely suitable and that a new referral would be needed in order to explore the options available. Since this time Steve has worked closely with the family and the Occupational Therapy department in order to make the room useable. It has since been agreed that the room can be reworked to make it more practical and suitable. After a number of meetings and regular communication we are at the point where the local authority is gathering quotes for the works including quotes for a height adjustable bath.

Greater London Advocacy: The Leathersellers' Company Charitable Fund

This grant has been particularly helpful for our advocacy service in the Greater London region, because it is ongoing over 4 years which gives us valuable continuity in planning our services. We have over 100 families living in this area, and we have provided wide-ranging support since this grant began in November 2009.

An example of support recently provided to a Greater London family: Michael and Josiah

Michael (aged 16) and Josiah (aged 13) have MPS IVA, Morquio disease. The family have been going through the process of adapting their home to the needs of both boys. They both get tired easily now but being teenagers they wish to be as independent as possible. Our Advocacy Officer Jolanta provided the family and the Local Authority with reports on the needs of individuals with MPS IVA as well as reports on the adaptations which might be required. Ongoing support has been provided through the decision-making and adaptation process, advising the family which adaptations may be better for the boys in the long run. The bathroom and staircase are in the process of being adapted.

When Michael turned 16 last year he had to apply for Disability Living Allowance (DLA) as an adult. The process of applying for DLA can be very stressful and upsetting for families as the forms are very complex and include a lot of questions focusing on the negative aspects of each individual's life and abilities. Jolanta supported the family with the completion of this form, and they were successful in achieving the higher rate of DLA for mobility and care. Michael was delighted about this, as it means he has been able to get a car, and has received help from Motability to have driving lessons. He has already passed his theory test and will soon be taking his driving test – we all wish him luck! It will mean so much to have the independence which being able to drive brings.

Michael's mum says: "Jolanta and the MPS Society have been really helpful. The DLA form was quite distressing, we were so grateful when Jolanta helped Michael. In these difficult times disabled people seem to have to fight even more for their rights and needs to be met, it can really wear you out so it is wonderful when you get the help, encouragement and advice from the advocacy workers."

Michael and Josiah with their brother Abraham and sister Rachel

Premex raises over £5000 for MPS

Premex, a Bolton based company, nominated the MPS Society as their 2011 Charity of the Year

Premex, a company based in Bolton recently presented MPS with a cheque for £5200. At the beginning of 2011 our employees picked the MPS Society for their charity of the year, this was following a number of nominations from employees. One of our employees, Edward Stringer, is very close to the charity as his daughter has Mucopolysaccharidosis Type I Hurler Disease and Ed is very thankful to the MPS Society and the work we do.

In 2011 Premex held numerous charity days – Easter, Halloween, cake sales and dress down days to raise over £5,000. We are very grateful to Premex and its employees for such generous support.

We would also like to thank Elizabeth Heath, mother of Jack who has MPS II, Hunter disease, who kindly represented the MPS Society at the cheque presentation held on Thursday 3rd May.

Elizabeth wrote to tell us how it went: "I would just like to let you know that I was honoured and proud to represent the MPS Society for the presentation.

I felt extremely privileged to be able to help out in such a small way and let people know all that you do for us as parents of one of these special children. Saying "thank you" seems so insignificant when you come out of your way to visit us at home and represent us "in our battles" with the authorities for what should be seen as needed and required to help take care of our loved ones - we should not have to battle for it and get stressed.

The fact that you get us together with other families for the conferences and fun days lets us know that we are not alone and we also get to help each other out as it is amazing what mines of information we become and specialists in our childrens' problems and where to source things from and how to get help for them - when we get together we can pass this on - even when only saying hello we can sometimes say something which will be of help to another family.

I personally feel that you will be our biggest support when we lose our son - you have a special bereavement service and unlike most charities, you don't just forget about us when we have no one to support us but you will be there for us when all the other people that have played such a large part in our lives will not - consultants, therapists, social workers etc.

Thank you once again and if there is anything else I can do to help and make a small difference please do not hesitate to let me know."

Coffee Morning - Cafe West, West Watford Free Church

The Cafe opened in September 2007, Tuesday mornings from 9.15am until 12.15pm. People from all walks of life come in and therefore we see and hear many different needs. We have all ages from those in push chairs to those in wheel chairs; those with physical disabilities to those who are fully able. Whether they come in alone looking for company or in a group for a social time together they come. Over the 4+ years we've been open we have had several fundraising mornings for the various needs that have been brought to our attention.

Tuesday 14th February 2012
MPS morning. We only recently become aware of MPS when a family, whose son suffers from the disease, started coming to the cafe a few months ago. It was not until Christmas however, when they were able to bring their son with them, that we saw the reality of MPS and how it affects not only the sufferer but the whole family.

As none of us had any in-depth knowledge or understanding of the nature of the disease we contacted the MPS Society. The website is extensive and gave us a tremendous amount of information but we needed to talk to someone. Following a phone call we were sent a full fund raising pack which included posters, leaflets and balloons: everything we needed.

On the day donations received came to a total of £211.17, an all time record for the morning. As all donations are on a voluntary basis, there is no set price on anything, this said much about the people who come along. It also said that the information given by the MPS Society had reached its mark. Thank you to the MPS Society for all you do. Below is a photo of a cake which was raffled. **Lesley Patterson** Social Secretary

Virgin London Marathon 2012

Go Uncle Rossy!

Ross Silock, uncle to Bobby who has MPS III Sanfilippo disease, ran the London Marathon in 4 hours 20 minutes and has raised over £2100 on his Justgiving.com page.

On the day all of my family were there to support Ross (they'd had T-shirts printed with "Go Uncle Rossy" on the front which he didn't know about until the day) including Bobby (his nephew) and his mum (my sister), dad and younger brother Tommy. They all had a fantastic day.

Jeremy Segal raised £973.50 on his Justgiving.com page through running the London Marathon for MPS. On his page www.justgiving.com/jeremymarathon2012 he writes: *'I am running for the MPS Society as I know a family who have been devastated by this and lost two lovely young daughters. Anything that can be done to help any families in the same position and go towards helping to stop it happening in the future must be good.'*

Marathon run in memory of Amie

Mark Sykes ran with a friend, Clare Brewster, raising £390.99 in sponsorship monies for the MPS Society plus £803.78 on his Justgiving.com page.

Clare raised £1211 on her justgiving.com page. Clare ran the marathon in memory of Amie Oliver. Clare writes on her page: 'Amie would have been 21 in March this year so to show her we haven't forgotten her (and never will) it seems a good time to raise some money in her name for the charity that supported her and her family. As all those who ever met Amie will know, she was a 'once met never forgotten' young lady. She had a huge personality, an infectious giggle and I know any of you reading this and thinking back to time spent with her will be smiling. She lived life to the full and despite being in pain and discomfort a lot of the time, she had an amazing sense of humour and was passionate about many things - especially Liverpool FC; her friends and family; pop music; and shopping to expand her very extensive clothing and jewellery collection - mostly pink, furry and/or glitzy!

Wherever she is I know she'll be laughing at me for running round getting sweaty in odd clothes, but she'll also be shouting the loudest above the crowds - "Come on Clare. Get on with it. Go faster..!"

Andrea and Louise's Marathon challenge

Andrea Parker and Louise Baker ran the London Marathon raising £2700 on their Justgiving page www.justgiving.com/andrea-parkerandlouise-baker.

In April 2010 my little niece Tillie Mae aged 2 was diagnosed with Sanfilippo MPS III sadly there is no cure for this disease. I couldn't just sit back and do nothing, so I made a brave decision to take on the challenge of running the London Marathon, admittedly being drunk but knew I would step up to the challenge as this is so close to my heart.

After I announced to my close friends what I intended to do, Andrea Parker a close family friend offered to join me on my challenge. Running was new to both of us so we found it very hard to begin with but watching Tillie Mae facing the tough times she had to deal with encouraged us to continue with this hard challenge. Running a marathon is nothing compared to what she has to deal with.

We started training even before we knew we had a place as we were determined that we would run the London marathon in 2012. We trained three to four times a week, fitting it in around our own busy lives as we both have families. We trained in all weather conditions with lots of 6am starts. Some days were very hard and others easier. During our training there was a lot of laughter and tears!

After months of training, marathon day was finally here, we were very nervous and excited. We had so much support throughout the day seeing familiar faces all along the route. This spurred us on to run each mile with a smile.

It was the moment that we saw Tillie Mae at mile fourteen, it was a magical moment and very emotional for everyone. It really hit home as to why we were doing this and made all the training worthwhile.

We raised lots of money for the MPS Society and were very grateful to all the kind people who sponsored us. We never imaged that we would raise as much money as we did for Tillie Mae and all the other children who suffer from this illness and also raising awareness for this awful disease.

It was an experience we will never forget and we hope that our contribution will help towards finding a cure to help our special little girl and all the children that suffer with this cruel disease.
Louise Baker and Andrea Parker

RGS 5A Sponsored Cycle

Damon Elson, Andy Day William Harrison, Tom Campbell, Rob Platt, Ben Moore, Ben Philipp and I decided to do our bit for charity. A charity that has always been close to my heart is the MPS Society that helps thousands of people thoughout the world who suffer from mucopolysaccharide diseases, very rare conditions.

I suffer from MPS II or Hunter Syndrome, so I was inspired to do my bit to support the charity to repay them for the help that they have given me and to give other children, with similar conditions to me, the chance to have the best opportunity of living a normal life.

Your donations will help the charity in many ways, these include: to provide support to individuals affected by Mucopolysaccharide and related diseases, their families and the professionals who care for them; to increase awareness of Mucopolysaccharide and related diseases amongst health care, social care and education professionals; to extend this to the community at large to secure improved local services and inclusion for affected children and adults and their families; to raise funds for research into Mucopolysaccharide and related diseases that may lead to improved clinical care, quality or life and new therapies.

This is a very worthy cause that are providing people like me with a better shot at life through Enzyme Replacement Therapy (ERT) (of Elaprase® for MPS II and Aldurazyme® for MPS I) and Bone Marrow Transplants etc.

Please do donate and make a difference to someone's life, by doing so there can be "Care today" for sufferers and "Hope Tomorrow" for their future.
Louis Garthwaite (MPS II Hunter)

The team raised £3735.19 on their Justgiving.com page!

Sponsored 10 mile walk for MPS

Kim Coney, her daughter and two of her sisters in law completed a sponsored walk over 10.5 miles from Lincoln to Wellingore on Saturday 5th May 2012.

They set off at 9.30am, and had some slippy slidey moments across parts of the Viking Way. Luckily the rain didn't lash down, just a few showers here and there. The tempature was more like March but it was enjoyable and well worth it of course.

We stopped briefly for a bite to eat once we got to Coleby, then marched on to the end which was a pub in Wellingore called the Red Lion.

They raised £205 in sponsorship monies. **Kim Coney**

MPS Charity Places available on events for 2012

For more information and to register your interest
please email **fundraising@mpssociety.co.uk** or phone **0845 389 9901**

BUPA Great North Run	16 September, Newcastle-upon-Tyne,13.1 miles
BUPA Great South Run	28 October , Southsea, Portsmouth, 10 miles
BUPA Great Birmingham Run	28 October, Birmingham City Centre, 13.1 miles

For more information about each of these events, please visit **www.greatrun.org**.

Running isn't just for adults - it's for kids too!

There are a number of children's races featured in the BUPA Great Run series.

For more information visit www.greatrun.org/junior

justgiving
fundraising made easy

Are you taking part in a sponsored challenge or event.
Use **www.justgiving.com** to encourage online donations.

The MPS Society has teamed up with justgiving.com so all our runners and events' participants can raise money quickly and easily online with their own personalised web pages. For more information visit **www.justgiving.com** or contact them as detailed here.

The JustGiving helpdesk is now available every day of the week.
If you or your supporters need expert fundraising advice, support and more, we'll be there, ready and waiting.
t: 0845 021 2110
e: help@justgiving.com

Team Lever on the Run!

The Lever family are raising funds for the MPS Society which supports Aaryanna and her family with her condition and the effects on their lives. On their Justgiving.com page, http://www.justgiving.com/Hayley-Lever0 they write:

"Our Challenges: Hayley is running (and perhaps walking, scrambling and crawling) the 21 mile Edale Skyline Fell Race on 25th March (apparently a "classic and often gruelling race"!).

Helen, Aaryanna and Elizabeth are running/walking the Newry 10k, on April 15th."

They have raised £401 on their justgiving.com page.

Jamie's sponsored cycle

Every year in our home town we have a festival week and one of the activities organised is a family bike ride. Jamie wanted to start doing the ride last year at age 5 just as his brother Matthew had done but Jamie has MPS I and the bike ride is approximately 5 miles.

Jamie however loves riding his bike so it was arranged that he would start and finish the bike ride – his Grandad would lift him in the car a short time after starting and take him close to the finish line to complete the race. However, when we got to the starting line we were told that the route had had to be changed which meant that he would have to complete the entire 5 miles as there was no access to the route by car.

I explained this to Jamie but he was undeterred so Jamie, his Auntie Noreen and I set off. I worried about him the whole ride wondering was it too much for him especially when I saw one of his school friends being pulled in a trailer behind their dad's bike. It took a bit of encouragement at times to keep him going but he did brilliantly. Jamie was determined to complete the ride and he did it – I'm so very proud of him.

I would like to say a big thank you to his Auntie Noreen and the employees of Dennison Commercials, Ballyclare who kindly sponsored Jamie so generously by raising £300.00 for MPS.

If you're taking part in a run or sporting event in aid of the MPS Society, please ask us for one of our **MPS running vests**. They are white, with the MPS logo, web address and Care Today, Hope Tomorrow strapline. Please help us raise awareness…
fundraising@mpssociety.co.uk

Bob Baines

I ran in the Brighton half marathon on 19 February 2012. I did this to raise money, for my Great Nephew William and his charity.

Many thanks for your support.

Williams story
William is 3 years old and lives in Worthing, West Sussex. A year and a half ago he was diagnosed with a life limiting condition, known as Mucopolysaccharidosis, Hurler Scheie Syndrome (or MPS type I). It is caused by a deficiency of a protein, critical to the normal function of vital organs, including the heart and the brain. Life expectancy is usually teens to twenties and there is no cure.

Since finding out about William's condition in January 2010, he has undergone a number of operations and has a weekly treatment called Enzyme Replacement Therapy (ERT). This involves putting the missing enzyme directly into his body through a tube via a vein. William's parents were trained to carry this out independently at home to regain some normality for their family.

Recently William has developed Hydrocephalus (water on the brain) and Carpal Tunnel Syndrome in both wrists. Both conditions require additional operations. These latest complications led to a review and it's now believed that William is at the more severe end of the scale of his condition and the ERT treatment has not proved as effective as was hoped. Because of this his life expectancy could be shortened even further and it is now recommended that he would benefit greatly from a bone marrow transplant. This gives his body the opportunity to produce it's own enzyme, enabling him a far better quality of life as well as increased life expectancy.

William's family are now preparing themselves for the bone marrow transplant, which will include at least two months in hospital with regular follow-up appointments in either Great Ormond Street or Manchester Children's hospital.

Considering all he has had to deal with in his early life, William is a happy, cheeky and loving little boy.

Latest Update
The great news is that William's Sister (Grace) is a full match for his bone marrow transplant. She has been a little trouper in having two sets of blood tests to determine compatibility and we are incredibly proud that she is willing to go through the donation process to help her brother.

Bob's Justgiving page,
www.justgiving.com/Robert-Baines
has raised £225.

Roger Harris ran the Brighton marathon on 15 April 2012 raising £521 on his Justgiving.com page for the MPS Society.

Melanie Campbell's daughter did a sponsored walk in aid of the MPS Society and raised £30.

Maria Murphy, mother of Tara, MPS I, ran in the Bath Half Marathon on 11 March raising £405 in sponsorship for the MPS Society.

Congratulations to **Andrew White** who raised £165 on his Justgiving.com page through a sponsored cycle from Land's End to John O'Groats. www.justgiving.com/bassyandflyawaylejog

Michael Krause raised £183 on his Justgiving page in sponsorship from the Great Manchester Run www.justgiving.com/Michael-Krause

Joanne Haines and Nicky Cato took part in the Great Manchester Run and raised £183 in memory of Lewis Cato who suffered from MPS I and passed away in November 2011. Their Justgiving page raised £183 in sponsorship www.justgiving.com/Joanne-Haines0

Edgard Zaldua, father of Daniel who has MPS III Sanfilippo, raised a further £1607.63 in sponsorship monies from his marathon run. This money is to go towards research into MPS III Sanfilippo.

George Bell who has MPS II Hunter's and is 5 years old ran the 1.5k mini run in Manchester on 19th May 2012. He was a star and ran all the way! George's mum Caroline ran the Manchester 10k. She did it in 62 minutes which is 2 minutes faster than last year. Between them they have raised over £250 on justgiving so far!

Tandem Skydives for MPS

Ellie and Stuart did a Tandem Skydive on 28th of March 2012 at the Langar Airfield Nottingham in Aid of the MPS Society. The Society does vital work supporting children who have this disease, and their families. They have raised £90 on their Justgiving.com site plus a further £421 in offline collections.

Thanks to Alison Oakins who raised £585 on her Justgiving page, www.justgiving.com/Alison-Oakins. Alison did a Tandem Skydive on 28th of March 2012 at the Langar Airfield with others including Kimberely Morgan, Colette Whittle, Gillian Ford, Carol Bancroft, Michelle Fox and Stuart Walker and Ellie Kennedy.

Sponsored Cycle for MPS

Peter Conlin's sister, Stephanie Thomas, did a sponsored cycle ride over 202 miles from Edinburgh to Newcastle for the MPS Society raising £310. Thank you to staff and pupils of St Cuthberts Primary School Hartlepool.

Peter's son, Ben, has MPS I Hurler Scheie. Ben is pictured here with Stephanie holding a cheque for the monies raised.

Pyjama Walk inspired by Tracey

My name is Marcia Burnett. I am 34 and from South Shields, Tyne and Wear. After complications at birth and being hit by a car at a young age my sister Tracey Underwood (now aged 37) had learning difficulties (or so we thought). She attended a mainstream school for a year or 2 until the Headteacher thought it was no longer suitable for her then she was transferred to a SEN school. Tracey had a relatively normal childhood, competing in cross country races etc and stayed in education until 19 years old then went on to adult training centres. Around 1998-99 Tracey was starting to show symptoms of MPS although at the time we didn't know what is was. After tests in 2000 Tracey was diagnosed with MPS IIIA (Sanfilippo) aged 25.

After seeing what effect it has had on Tracey over the years I was inspired to raise money and awareness for MPS and related diseases. I arranged a 5 mile Sponsored Pyjama Walk on May 13th. Friends and family (19 of us) got involved, even my 78 year old nanna in her wheelchair! We all had fun on the day, in our pyjamas we walked from our local leisure centre down to the coast road, stopping off at Minchellas for an ice cream along the way before heading back on another route to the leisure centre, our finishing line. We raised an amazing £670.50 for the MPS Society so it was well worth it.

EVENTS & CHALLENGES

Are you thinking of participating in a sponsored challenge or organising an event? Read on…

Find us on Facebook

Sponsored fundraising events are very popular and an easy way of raising money. We can supply promotional materials to support your event including posters, balloons, stickers, t-shirts, collection boxes and buckets. Set up a page on justgiving.com so that people can donate at the click of a button.

You can be sponsored for almost any type of event. You may like to try a walk, swim, or something a little different such as holding a tea-party, getting sponsored to shave or jumping into a bath of baked beans. If you work for a company, ask them to match funds received pound for pound that their employees raise for registered charities. The more original or difficult your event, the more money you are likely to be sponsored! For example, giving up smoking or even chocolate, giving a five minute massage in your lunch break, holding a quiz night, sponsored slim or a fancy dress party! Ask yourself whether there is anything you have always wanted to do, or wanted to give up. Do you have a particular hobby that is interesting and fun?

Try to get some coverage in your local media. They like to feature inspirational stories so let them know about your event. This will raise awareness of both MPS and your event or organisation. If you need to write a press release or would like some advice talking to the media do give us a call. There is a sample press release available as a download from our website.

Make use of your local amenities, for example, local pubs, restaurants and shops as they are great places for holding events, displaying posters and promoting awareness. Check whether you need permission from anyone to use their venue. Ask small companies to donate gifts as they will benefit from the publicity and supporting worthwhile causes. An excellent place to begin your fundraising is at your place of work. Use staff noticeboards, pigeonholes and email to spread the word. If you belong to any clubs or societies, get them involved.

You can do your sponsored event on your own as an individual, or you could persuade your friends and relatives to take part with you. This may be more fun and you could raise even more money if there is a group of you. Those who want to get involved, but who do not want to actually take part, may be willing to take a sponsor form to their school or workplace. Never feel guilty asking people to sponsor you.

Once the event is over, you need to start collecting the sponsorship money, chase any late donations and total up the final amount you have raised. Send it to us along with the sponsorship forms and a story and photo for our fundraising magazine.

Ask us for a fundraising pack which contains more information and points to consider. Phone **0845 389 9901** or email **fundraising@mpssociety.co.uk**

Health and Safety is an important issue that must be considered whenever you arrange an event. Here are a few things to bear in mind.

The Society does not authorise organisers of fundraising events to act as agents of the Society.

Whilst the Society appreciates your support, it is not responsible for organising, supervising or hosting your event. Individuals taking part in activities do so at their own risk.

We do not accept any liability for your event, loss or damage to yourself, property or personal effects, so if you need insurance you must arrange this yourself. Inform your insurance company in plenty of time.

Please do not do house to house collections. The Society does not support this. Please be aware there are strict rules and regulations which you must observe. Please ensure you have made yourself aware of these and follow them.

Health and safety is a serious issue. Give your local Red Cross or St John's Ambulance a call if you intend to organise an event.

Supervise children and don't let them collect money from strangers.

Only get sponsored by and collect donations from people you know and trust.

No one under the age of 16 should be collecting sponsorship money without adult supervision.

Children should always have permission from a parent or guardian to participate in a fundraising activity.

Always wear safety equipment if required.

Ensure you have enough volunteers to control the crowd that may arise.

Be careful if you are carrying money. Put it in a safe place and keep it out of sight.

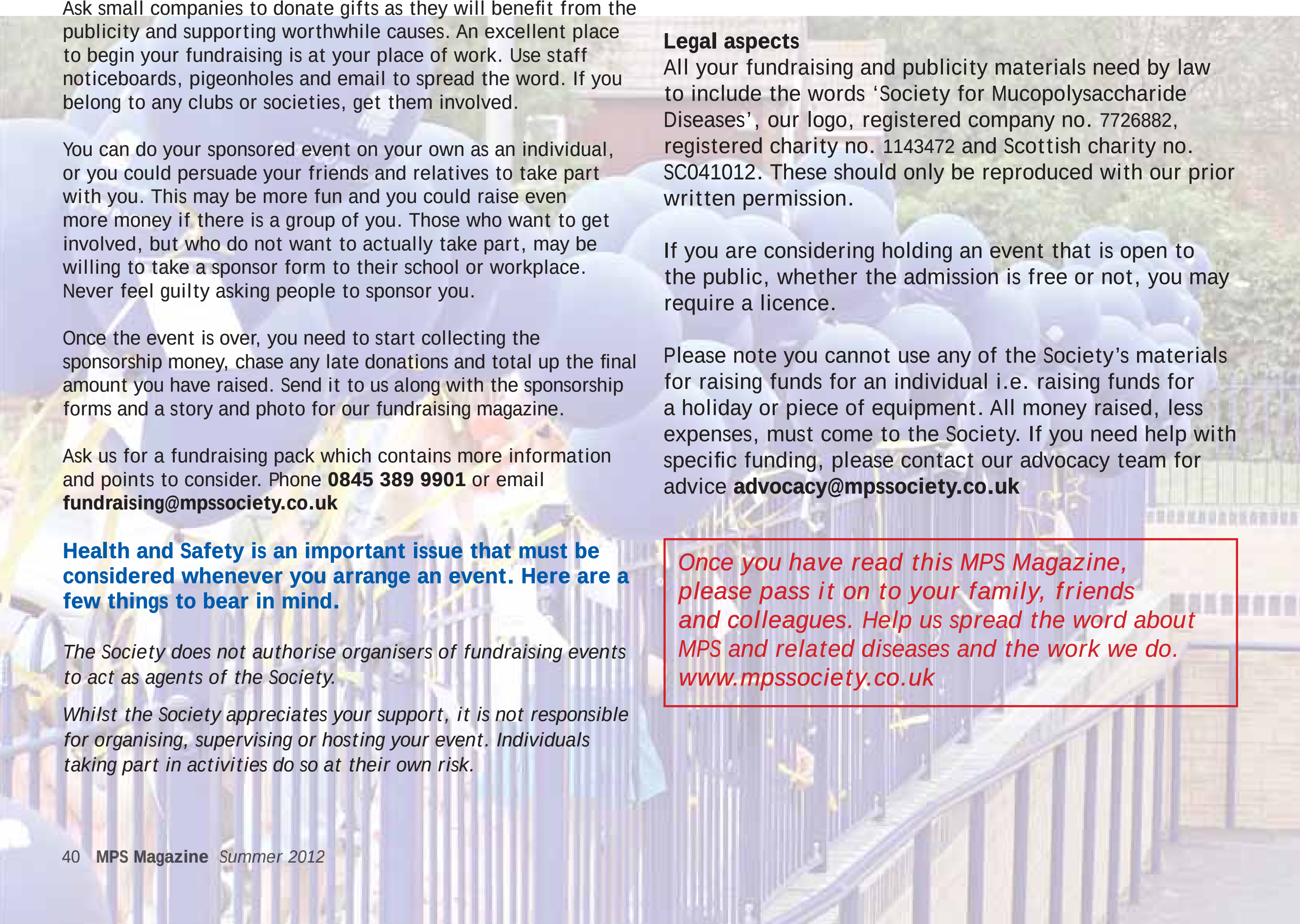
Legal aspects

All your fundraising and publicity materials need by law to include the words 'Society for Mucopolysaccharide Diseases', our logo, registered company no. 7726882, registered charity no. 1143472 and Scottish charity no. SC041012. These should only be reproduced with our prior written permission.

If you are considering holding an event that is open to the public, whether the admission is free or not, you may require a licence.

Please note you cannot use any of the Society's materials for raising funds for an individual i.e. raising funds for a holiday or piece of equipment. All money raised, less expenses, must come to the Society. If you need help with specific funding, please contact our advocacy team for advice **advocacy@mpssociety.co.uk**

Once you have read this MPS Magazine, please pass it on to your family, friends and colleagues. Help us spread the word about MPS and related diseases and the work we do. www.mpssociety.co.uk



The Book-keeping Beekeeper

One beehive, forty thousand bees, eighty jars of honey and the odd sting or two. That's the summary of my 2011 beekeeping season.

Having enjoyed a beekeeping beginners course at River Cottage HQ some years ago, I've been fascinated by the world of the honeybee ever since. It's been an interesting voyage of discovery. Lots to learn and plenty of new terms to comprehend; Commercial, Langstroth, National, Dadant, smoker, excluder, super, foundation, frame, skep, extractor; and that's before we even consider the bees themselves! I've also discovered that if you ask three experienced beekeepers for advice, you will invariably receive five different opinions!

With the help and friendly support of the West Hertfordshire Beekeeping Association, I now have a strong colony established at the end of the garden.

Last summer, I struck on the idea of trading some jars of honey for donations to the MPS Society. This has been very successful and I would like to thank all those who have placed a donation on my justgiving.com web-page.

As we move into the 2012 season, I am now in the process of starting up a second hive. Fingers crossed that we have another bumper honey crop and a few more donations to add to the fundraising total.

So, for those that are counting; that's two beehives, eighty thousand bees, lots of honey and maybe the odd sting or two more.

Malcolm Puntis, The Book-keeping Beekeeper at Hillcroft Honey has raised £215 on his justgiving.com page, www.justgiving.com/Hillcroft-Honey.

Thank you to…

Liz Lee recently completed her tenure as chair of the Riviera Tangent. They are based in Torbay and are old Ladies Circlers. As outgoing chair Liz was allowed to present a cheque to a charity/charities of her choice. She very kindly chose to donate £50 to the MPS Society.

Elizabeth Mee held a coffee morning for friends and neighbours raising £100 for the MPS Society. Elizabeth says that they were more than happy to support the MPS Society knowing her grandson, Sam Brown, would benefit from our work. Sam, who has Morquio, is currently taking part in the trials at Manchester Children's Hospital. Elizabeth says that her friends so enjoyed themselves that they asked if she would hold another coffee morning to raise even more money!

Carol Bancroft donated £203 to MPS being the proceeds of a bucket collection at a local bingo hall following her skydive for the MPS Society.

The Worldly Goods Shop

Susan Radford, Treasurer of the Worldly Goods Shop, kindly donated £500 to the MPS Society.

She writes: "We are a small retail business promoting fair trade products, run by volunteers. Our aim was to break even each year but this normally meant we made a small profit which we distributed to charities we felt had similar ethical ideals to our own. We never gave money to the same charity one year after another but simply relied on our volunteers for their ideas.

After 30 successful years of helping the local community see just how important fair trade is, we have closed our doors and left fair trade to the wider retail world. That therefore gave us a small amount to distribute among half a dozen charities. One of these is the MPS Society which was chosen because of a personal contact of one of our volunteers."

Thank you to Marina and Friends

We would like to extend a special thank you to Marina Foster and friends. Marina runs a charity shop in Bristol, Marina and Friends Fundraisers, donating the proceeds from the sale of second hand items to the MPS Society. So far, the cumulative total raised by Marina and Friends for research into Sanfilippo disease is **£80,149.29**. If you would like to support the MPS Society by providing items for Marina to sell, please find below the address for the shop:

Marina & Friends Fundraisers, 44 Sandy Park Road, Brislington, Bristol, BS4 3PF.

You can also follow Marina and Friends Fundraisers on facebook.

WAYS TO SUPPORT US AT SCHOOL

For our **School Fundraising Pack** and other fundraising materials visit **www.mpssociety.co.uk**

Studens of 11A at **Dover Grammar School for Girls** held a cake sale event and raised £44.56 for MPS.

Ysgol Gynradd Gymunedol Penparc Community Primary School raised £175 for the MPS Society. The school council collected the money as they have a young girl in school who suffers from MPS.

Sandford St Martin's First School supported MPS Awareness Day raising £238 for MPS through their contact with Josh Cutler who has MPS II Hunter disease.

Hannah Talbot in 9 Orange of Davison CE High School for Girls held a raffle and cake sale raising £72.80. Hannah wanted this money to go towards the work of the MPS Society.

Pop Idol concert for MPS

Sharples School in Bolton donated £700 being the proceeds of a Pop Idol concert in aid of the MPS Society. The school were made aware of the Society through Ian Pulston-Davies (Owen from Coronation Street) and 5 year old Gracie Mellalieu. Gracie and her parents attended the evening.

The photo to the left is of the 8 contestants, Gracie Mellalieu and her Mum and the three judges - Ian Puleston-Davies (Owen Armstrong in Coronation Street), Tower FM's Jon Holling and Bolton Music Service's Jamie Taylor.

Don't forget to check out the fundraising section of our website for further information and ideas www.mpssociety.co.uk

Giving Calcs

By Aura Creative Communications

Open iTunes to buy and download these apps.

A simple, easy to use tool to calculate the cost and value of tax-effective gifts.

This app, commissioned by the Institute of Fundraising, is designed for both donors and charities alike to calculate the benefits for different forms of tax-effective Giving.

The calculators include:

- Gift Aid
- Share Giving
- Payroll Giving
- Legacy Wealth

For any further information, please visit **http://www.tax-effectivegiving.org.uk**

Donate by Text

The Society for Mucopolysaccharide Diseases supports individuals and their families affected by MPS and related diseases throughout the UK.

We rely on your generous support to enable us to provide a unique advocacy support and information service including access to clinics, conferences and events.

Donations can be made online at www.mpssociety.co.uk.

We are also now delighted to accept donations by text...

Text MPSS01 £2 / £5 / £10 TO 70070 to donate now!

Thank you!

JustTextGiving by vodafone

WAYS TO SUPPORT US AT WORK

Fundraising at work is a great way to boost your employer's reputation with shareholders, suppliers and the community. It also provides your colleagues with a great chance to get to know each other! Check out www.mpssociety.co.uk for more information but here are some ideas…

- Dress up, or down, or choose a fancy dress theme - those who take part donate £1, those who don't pay £2 (as a penalty for not getting involved)
- Guess the baby competition - get everyone to bring in a photo of themselves from a particular decade, or under the age of 3 for example, and pay to guess who each photo is of. The person with the most matches wins
- Pack your lunch - save money by bringing in your lunch instead of buying it, and ask people to donate what they would have spent into the collection pot. Designate a week to do this and watch how the money builds up!
- Swear box - any container will do. Every time you or a colleague swears on a designated day means that a £1 penalty must be paid
- Use the stairs - have a forfeit box near the lift and ask people to make a donation every time they give in and the use the lift
- Abandon your car - get sponsored to walk or cycle to work
- Guess the number/weight - either fill a jar with an item and ask people to guess the quantity or display something and ask people to guess the weight
- No email day - get everyone walking and talking and impose a penalty fine for those who cheat
- Ask us for an MPS Collection Box and have this on display so you can collect up all that loose change
- Recycle old mobile phones, ink cartridges - ask us for a freepost envelope
- Send us used old stamps from incoming post - we can exchange these for money to MPS
- Plan a sponsored challenge such as a skydive, cycle ride, trek or run with a group of colleagues. Ask us for a fundraising pack or check our website for the latest opportunities **www.mpssociety.co.uk**

Many larger companies now operate a **matched funding scheme**, whereby they will match all the funds raised by one of their employees up to a specified limit. So, if you raise £200, you may find that your company will match that money with another £200.

John Patrick at Savills plc donated £2227.50 through the Charities Aid Foundation.

Can you help us gain support from companies?

In these challenging times the MPS Society is looking to increase the number of companies that we talk with to gain their financial support and we would really appreciate your help with this.

Many companies have a positive attitude to supporting charities that their employees are involved with. Some of the better known ones are Admiral Group, Best Buys, Carphone Warehouse, John Lewis Group, Waitrose, Marks and Spencer and many of the banks. These are the tip of the iceberg and we are asking that if you, a family member or friend are working for one of these companies or indeed any other company that we could be nominated to the charity committee.

The selection process, although different for each company, requires a staff member to nominate their charity. Sometimes the nomination process is simply emailing the name of the charity (Society for Mucopolysaccharide Diseases) to the Committee. However, if they require supporting information please contact **fundraising@mpssociety.co.uk** or phone **0845 389 9901**.

If you have any stories, advice, news or pictures that you would like to share with us, please do send them in. We will return any original photos as soon as possible. Your comments and suggestions are always welcome. **fundraising@mpssociety.co.uk**

Here at the MPS Society we love to support fundraising ideas that you may like to take on, or to hear of any ideas that you think we should be considering. The MPS Society is only able to exist and develop with your help so please do contact us by email at **fundraising@mpssociety.co.uk**. Thank you!

Project Sanfilippo

The concept of Project Sanfilippo was developed by Karen Robinon, mother of Oliver who has Sanfilippo. Project Sanfilippo raises vital funds for the MPS Society to put towards research into Sanfilippo Disease. On this page we introduce Project Sanfilippo...

My 13 year old son Oliver has Sanfilippo Disease (MPSIII) Type C and we have been members of the MPS Society since Oliver's diagnosis in 2006.

I, and my husband, Stuart, along with a small group of friends managed to raise £30,000 to convert our garage into a sensory and soft play room for Oliver. In the future it will become his bedroom when his needs change with the progression of the disease.

A lot of the money was raised from an Auction of Promises from which we raised £13,000. Now that we have adapted our home for Oliver, we intend to focus on raising funds for research into Sanfilippo Disease and raising awareness of this disease.

In 2010 I launched "Project Sanfilippo" (working jointly with the MPS Society) with our first Taste and Tell event which raised £850. In September 2011 we held our second and now hopefully annual Taste and Tell, raising a further amazing £2,226.

The money raised by Project Sanfilippo will go to the MPS Society for them to restrict entirely towards research into Sanfilippo Disease. Any gift aid that can be claimed back on eligible donations will go towards the general running costs of the MPS Society.

When we set about raising the shortfall for Oliver's Room many of our friends said how delighted they were to actually be able to do something tangible to help us, as generally there is not much people can do to directly support Sanfilippo families. I feel very fortunate and grateful for all my amazing friends as well as The MPS Society and all the research the scientists do for all the MPS families. This happens without ANY government help.

I am inspired to do anything that will hopefully help Oliver and other children with Sanfilippo all over the world.
Karen Robinson

Monies raised through Project Sanfilippo will be put towards research into MPS III Sanfilippo disease.

Several events have been held through Project Sanfilippo raising funds for research into Sanfilippo disease totalling over £4000.

These have been published in past editions of the MPS Magazine and we are delighted for this support into vital research into MPS III. A number of individuals also have events, challenges and fundraising activities planned for the coming months and we will feature their stories in subsequent MPS Magazines together with articles from St Bartholomew's School in Berkshire who have kindly chosen the MPS Society Project Sanfilippo as their chosen charity for the year.

Farmor's School Fundraiser

Every year in March Farmor's School in Fairford, Gloucestershire, club together and raise money for Comic Relief. However for March 2010 there was a twist – following recent events the Charity Committee decided to split the money between four charities – Comic Relief, The British Red Cross, Friends of Farmor's and Project Sanfilippo through the MPS Society.

I put forward Project Sanfilippo as my cousin Oliver has Sanfilippo disease and I wanted to help raise awareness as well as money for the charity. Therefore as school opened on Friday 18th March 2011 there was a flurry of activity – non-school uniform for Year 7-11 and fancy dress for sixth form (costumes ranged from pirates to the three little pigs to pyjamas) who all donated £1-£2 for the opportunity, as well as cake sales and students in sombreros with guitars singing and shaking buckets for donations.

All in all Farmor's managed to raise just under £400 for Project Sanfilippo, as well as spreading awareness of the disease. **Ashwina Ellis**

Further information
There will be further updates about Project Sanfilippo in future editions of the MPS Magazine.

To sign up to receive our MPS Magazine by email, please visit our website www.mpssociety.co.uk

Fundraising Standards Board (FRSB)
The MPS Society is a member of the FRSB. For further information please visit our website www.mpssociety.co.uk

www.mpssociety.co.uk

We have a wide range of information and educational resources available to either download from our website **www.mpssociety.co.uk** or to purchase through our **online shop**. You can access our online shop through our website. Alternatively, if you don't have access to the internet, please phone us for a current publication order form or MPS merchandise leaflet, 0845 389 9901.

From our website you can also subscribe to receive the quarterly MPS Magazine by email, check out other ways you can get fundraising and supporting MPS, a list of all the different ways you can make a donation **AND** find out how your money helps us…

Would you like to volunteer for us?

Volunteering is fun and rewarding. It could also help you learn new skills and gain valuable work experience. The MPS Society relies on volunteers for our events and conferences to assist in the care needed for children and young adults affected by MPS and Related Diseases.

All of our volunteers undertake training in moving and handling and are fully briefed prior to the event. Volunteers should be 16 years or over, will need to provide two references and undergo a Criminal Records Bureau check and attend a training day in Amersham. Those volunteering for our conferences will receive accommodation and all meals throughout the weekend.

Contact us now to register your interest and availability. **mps@mpssociety.co.uk**

Help us care for today and give hope for tomorrow, leave a gift in your Will

It is vital that the MPS Society has sufficient funding to be able to look forward to the future with confidence. One way in which you can support the Society achieve its long term objectives is to include the Society when drawing up your Will. For more information please contact us for our Leaving a Legacy leaflet or for more information please visit **www.mpssociety.co.uk**

Find us on Facebook

The MPS Society Facebook page is a means of providing information to our MPS Members and Friends quickly and efficiently. In the future we hope to feature some of our events and activities and recognise those that contribute to the Society and the work that we do.

You can find us by entering *MPS Society* into the facebook search engine.

As well as aiming to provide you with news from our fundraising activities and MPS events to coincide with our quarterly MPS Magazine we are also hoping to encourage greater awareness of the MPS Society.

If you have any ideas or suggestions for our facebook page please email **facebook@mpssociety.co.uk**

Do you have a story to share?
Please email
magazine@mpssociety.co.uk
or phone **0845 389 9901**

www.wickedgenes.co.uk

Gift Aid Q&A

What is Gift Aid?
It’s a scheme introduced by the government which allows charities like the MPS Society to reclaim the tax that supporters have already paid on their donations. For every £1 donated, the taxman gives us an extra 25p, so our income from your donations increases by almost a third. We can only claim on donations made within the last six years’, tax unclaimed before this will be lost forever, so please act now.

Why 25p per £1?
It’s linked to the current basic rate of income tax (which is currently 20 per cent). As a basic rate taxpayer, for every £1.25 you earn, you will pay 25p to the taxman and receive £1 in your pocket. It is this 25p we claim.

Am I eligible?
If you are a UK taxpayer and have paid enough income or capital gains tax in the financial year to cover the amount we’ll reclaim, then yes: please complete a Gift Aid declaration.

Where can I get a Gift Aid declaration form?
You can download a Gift Aid declaration form from our website or we can post one to you. Please complete one of these forms and return it to the address specified on the form. If you require assistance please call 0845 389 9901.

What if I’m a pensioner?
We can still claim the full 25 per cent on your donations, providing you pay at least as much tax as we will be reclaiming in the year in which you made your gifts. As a pensioner, for example, you may still pay tax on a private pension scheme or a savings account, or pay Captial Gains Tax if you sell property or shares.

What if I’m a higher-rate taxpayer?
We can only claim the basic rate back, but this is still extremely valuable. You will also be able to claim additional personal tax relief on your self-assessment form.

I’m not eligible; should I still return the form?
Yes please. It would be a huge help if you could return a declaration to us letting us know not to claim. This way, we won’t contact you about Gift Aid again in the future, saving us time and money.

I’ve already completed a declaration for another charity. Do I need to complete one for The MPS Society?
Yes. You will need to complete a separate form for each charity you want to benefit from Gift Aid.

What else does ticking the Gift Aid box commit me to?
Nothing at all. It just ensures that, if you donate to us, we can claim money back from HM Revenue and Customs.
How long does my declaration last for?
Until you tell us to stop. At the moment, and as long as you remain eligible, your declaration lets us reclaim past (up to six years), present and future donations.

What do I do if I need more information on Gift Aid?
If you would like to find out more, or if you would prefer to make your Gift Aid declaration over the phone, please contact us on 0845 389 9901 and we will do our very best to help.

What if I change my mind, or am no longer paying tax?
Please contact us either by letter to The MPS Society, or by e-mail to fundraising@mpssociety.co.uk, and tell us that you no longer wish us to claim the tax on your gift.

What if I’m an events participant?
If you have received a benefit by participating, although anybody can sponsor you, MPS Society may not be able to claim Gift Aid from all your sponsors, including family members (‘Connected Persons’). Please check with us for further details.

Will it really make a difference?
Yes. You will be increasing the value of your donation which will go the extra mile for those who need our support.

In the financial year for 2011 the MPS Society has been able to reclaim £3132.21 in Gift Aid.

Making a donation

By post: Send us a cheque or postal order. Don’t forget to enclose your address so we can reply and send you a thank you. Please don’t send any cash by post.

At a bank: Pay in your cash or cheque at any branch of Barclays. Our account number is 33986306, sort code 20-02-06. Just let us know who you are and what you’ve done!

By credit card: Phone us or visit our website to donate online. Please ensure you let us know your details and how you have raised the money. Most major credit cards accepted.

Online: You can donate online through our secure server by visiting **www.mpssociety.co.uk**

For other ways of making a donation, please ask us for our Making a Donation factsheet.

Please let us know when you are making a donation or paying in money directly into our bank account and please include a reference for the payment. This way we can confirm safe receipt of the funds and say a big thank you. If we can’t identify where the funds have come from then we can’t thank you! **fundraising@mpssociety.co.uk**

SPECIAL THANKS TO…

Sheila Campion at the **Christadelphian Sunday School** sent in £66.66 to MPS being the proceeds of their annual collection.

Holly Nowell at 'Beryl' who didn't hold an open garden this year but kindly donated £120 instead.

Eira March organised a bring and buy sale held at St Mellon's over 50s coffee morning raising £100 for MPS.

Maureen Cockram, Treasurer for the **NHS Retirement Fellowship**, donated £100 to MPS being the proceeds of collections for MPS throughout the financial year 2011/2012.

Kate Evans donated £90 to MPS being the proceeds of a charity quiz at Haven on the Hill in Cwmfelinfach.

Family, friends and people of Abercarn who donated £325 in loving memory of Angelene Hayward following the sad passing of Kathleen Hayward.

Sara, a friend of Yvonne and Phil Pearson (parents of Archie MPS VI) kindly donated £50 to MPS.

Jenny Quant sent in cheques for £500 collected in memory of her mother-in-law Sheila Quant whose grandson Lewis has MPS III Sanfilippo.

Derek Wright donated £35 to MPS being monies he received from Gillian Kenny Associates for participating in a Fabry study.

Donna Bown sent in £22 being the proceeds of a table top sale at Asda Eastbourne.

Bill Forsey kindly donated £395 to MPS in memory of his wife, Pamela, who sadly passed away on 7 November 2011. Bill wrote that he and his wife were so grateful for the support provided by the Society to their family when their granddaughter Rhianneth Louise Wheeler was diagnosed with ML II at the age of 1 year and then the comfort and help received when she later died aged 3 years.

Dinah and Damien Adair donated £2000 to the MPS Society having been inspired by the stories of Roma and Charlie at the MPS and Fabry Irish Conference. Thank you for your continued support.

Susan Norris donated £100 as a wedding gift for Mr and Mrs Des Hope.

Pam and Ken Ballard kindly donated £10 in memory of Simon Lavery.

Ann Parsons has raised £73 through the sale of MPS trolley key rings at Asda Eastbourne.

Judy Holroyd donated £10 being donations from surplus greenhouse plants.

Avril Earnshaw and her Slimming World group kindly donated £95 being the proceeds of a Christmas raffle at the sessions she runs. Thank you to everyone who kindly donated.

Tesco Stores Ltd, Coventry, kindly made a donation of £115.50 in memory of Daniel Singh.

Stamps, foreign coins, mobile phones, ink cartridges, jewellery: J Stather; Langlea House Care Home; Timsbury Wine Circle; Dorothy and Colin Robinson; Elizabeth Heath; Mrs M Brock; Marilyn Eggleton; Letitia Ricketts; Ian Evans

Donations: Alan and Monica Bowen; Shabana Kausar; Mrs Elanapa; Mrs P A Todd; T & E Mason; G W Swift; Barbara Challen; E M Slingo; Mr and Mrs Crespin; Joy Beaverstock; R D Challen; Ian Puleston-Davies; Liz Merryweather; Tracey Evans; Claudia Centanni; Helen Goff; Michael J Newell; Pam and Ken Ballard; George Tsang; I and J Sanderson; Pat Gordon; Haddenham W.I.; Mike and Janet Hynes

In memory: Shujah Altaf; Valerie Challen; Simon Lavery

Collection boxes: Sue Hollidge

The Society would like to thank the following donors for their regular contributions by either Standing Order or Give As You Earn: M Kalsi; D J Holmes; P Summerton; J Dalligan; M Malcolm; E Mee; K Brown; M Fullalove; M Reeves; McCann; R Parkinson; R N Taylor; R & K Henshell; K Bown; S & J Home; V Little; L Wood; E M Lee; Katy & Simon Brown; J & V Hastings: S Littledyke; R Dunn; K Seeber; S Bhachu; C Cullen; G Simpson; William Cavanagh; L Brodie; E M Brock; E P Moody; S & D Greening; Evelyn White; C L Hume; A Weston; A Sullivan; A Byrne; Mrs Robinson; N & S Cadman; J Wilson; J York; M Wood; E M Cox; Molly Rigby; K Robinson; K Osborne; Mr Thompson; C Lunnon; A Tresidder; J Casey; C Garthwaite; S J Hill; M J Peach; R Arnold; J H Ellis; I Pearson; D & S Peach; Andrew Cock; A Dickerson; C R Gibbs; Norman Saville

Requesting more information by post

If you would like to receive more information by post, please tick the appropriate boxes, complete your contact details and return the form to us in the post.

☐ Fundraising Pack
☐ School Fundraising Pack
☐ Publication Order Form
☐ Merchandise Order Form
☐ Ink cartridge re-cycling Envelopes
☐ Jewellery Recycling Envelopes
☐ Cardboard Collection Box
☐ Gift Aid Form
☐ How to make a regular donation
☐ Leaving a Legacy leaflet
☐ Payroll Giving
☐ Information on becoming an MPS Childcare Volunteer

Name: ………

Address: ……

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Email: ………………………………………………………………………………………… Tel. No. ……………………

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