MPS

Spring 2012

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Society for Mucopolysaccharide Diseases

Please donate to
www.mpssociety.co.uk,
phone 0845 389 9901
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: Isabel Annakin (MLD)
See page 10 for more information

Society for Mucopolysaccharide Diseases

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Registered as a Company limited by guarantee in England & Wales No. 7726882
Registered Charity No. 1143472
Charity registered in Scotland SCO41012

Magazine Deadlines

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

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

IN THIS ISSUE...

Spring 2012

Welcome to the Spring 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.

Turn to our fundraising section for stories from our wonderful supporters, raising money to keep our support services going. There are plenty of ideas to inspire you to help us raise money! Don't forget that MPS Awareness Day takes place on 15th May. Can you help us to spread the word about MPS?

We also include our Children's Newsletter as a centre spread. Please do send in your stories and photos, everyone loves to read them! If you would like to make suggestions for what we can include in this section, please drop us a line!

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

Important Message from the Chief Executive

As I sit down to write to you I am conscious that many of you are facing additional burdens related to the challenging economic situation of this country and beyond. Whilst the Society can not make everything right for everyone it is at times like this that I would encourage you to ensure you are getting all the help you can, particularly in terms of disability benefits and costs of travelling to hospital appointments. Please remember that the advocacy team are only a phone call or email away if you feel you need help or advice. I must however caution you that a handful of our members have recently found themselves in difficult circumstances legally for unintentionally not telling the Department of Work and Pensions of changes in circumstances in respect of Carer's Allowance.

So how is the Society faring in these difficult economic times? Well at the Society's year end it had a small surplus of £92,277 however in the first few months of the new financial year we have seen a much reduced level of income and virtually no new money has been received. Prudence has to be the word whilst finding economies that will have little impact on our members and the services we offer. In the last MPS Magazine we said goodbye to Jolanta Turz after over three years as a member of the advocacy team following her decision to return to her native Poland. After discussions with Trustees and the Senior Management Team the decision was taken not to recruit to this vacant post until we could secure new funding for at least twelve months. We are also sorry to see Lindsey Wingate leave us to give more time to her family and are busy recruiting to her post. We are working very hard to secure this funding for Jolanta's post but it may be some time before we are up to our full complement of advocacy workers. In the meantime Rebecca Brandon, Steve Cotterell and Alison Wilson supported by Sophie Thomas and I will be working even harder to ensure the advocacy service does not suffer.

Fundraising is core to the very existence of voluntary organisations and the MPS Society is no exception. Having acknowledged that these are very difficult times we would say that there has never been a year when the MPS Society needs the support of its members, friends and supporters more. Whilst focussing on keeping the Society's core activities, individual advocacy and support programme adequately resourced we are applying for restricted grants to hold family days, focus groups, sibling weeks, young adults weekends and regional and national conferences. We are pleased that we are able to host a regional conference in Northern Ireland in May but we could do a lot more if we had more money.

Since the MPS Society along with Great Ormond Street Hospital and the Primary Immunodeficiency Association were no longer Jeans for Genes partners as of March 2011 it has been extremely difficult to secure the major funding the MPS Society needs to support important research projects. A clinical trial to evaluate Genistein as a potential treatment for Sanfilippo disease at the University of Manchester is ready to start but without a further £800,000 patients can not be recruited. The MPS Society is steadily working towards the launch of a public fundraising initiative that we hope will generate the money we need to fund important research projects of which the Genistein clinical trial is a priority. Although the plan is to trial Genistein on children with MPS III if proven to show efficacy this product does have the potential for use in other MPS and related lysosomal storage diseases where there is neurological involvement.

On a different note, and as you will read later in the MPS Magazine, two clinicians known to many of you are on the move. Dr Uma Ramaswami joined Prof Ed Wraith and Dr Simon Jones on the LSD team at Manchester Children's Hospital on the 1 February 2012. Dr Chris Hendriksz will be leaving Birmingham Children's Hospital on 31 March 2012 to join the adult LSD team of Dr Reena Sharma and Dr Ana Jovanovic at Salford Royal Hospital where he will be using his experience in paediatric and adult metabolic medicine to care for young adults with MPS and Fabry and those patients transitioning from Manchester Children's Hospital. I am sure you would join me in wishing them both well in their new positions.

Christine Lavery
Chief Executive

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 Trustees' Board Meeting held on 26 November 2011.

Incorporation

The Chief Executive reported that she has received the final Articles and Memorandum from the Society's legal advisers and these will be circulated to Trustees.

Personnel

The Chief Executive reported that Jolanta Turz would be leaving the MPS Society to return to her native Poland after over three years as an MPS advocacy officer. Everyone offered Jolanta their best wishes for her future. Trustees supported the Chief Executive's recommendation that the vacant advocacy officer post should be frozen until restricted funding for the post is secured.

Clinical Management

The Chief Executive appraised the Board of the progress of the Transition Modelling Project and confirmed that all consultations have been completed. Due to a change in personnel a second consultation will be facilitated with one NCG LSD centre as soon as possible.

A discussion took place around Homecare for patients on Enzyme Replacement Therapy and the CEO confirmed that a new national tendering process is under consideration.

Advocacy

Alison Wilson spoke of her work as All Ireland Advocacy Officer.

Relationships With The Pharma Industry

The Chief Executive confirmed that David Meeker has been appointed CEO of Genzyme Sanofi based in Boston, USA. Since his appointment Mr Meeker has been engaging with the individual LSD patient associations. The MPS Chief Executive met with Mr Meeker on 4 November 2011 in London. Steve Cotterell, Advocacy Officer attended the Fabry Lounge Meeting hosted by Genzyme in Budapest in November 2011.

The Chief Executive presented the work of the MPS Society to staff of Shire Pharmaceuticals at their offices in Basingstoke as part of a fundraising lunch and learn for the MPS Society. The MPS Society continues to liaise with Shire as part of the Patient Access Programme for the MPS II and MPS IIIA Intrathecal Clinical Trials.

The MPS Society continues to liaise with BioMarin as part of the Patient Access Programme for the MPS IVA Enzyme Replacement Therapy Clinical Trials taking place in London, Manchester and Birmingham.

On 19 September 2011 Alison Wilson, MPS Advocacy Officer attended a Fabry Expert Meeting in Dublin organised by Amicus.

Fabry International Network (Fin)

The Chief Executive informed Trustees that she has been appointed Secretary of FIN.

Risk Management

The Chief Executive confirmed that she had reviewed the risk register in preparation for the Trustees' Meeting and confirmed that work in progress includes finalising the Staff Handbook and Disaster Recovery Plan.

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

News from the MPS office

Introducing **Jo Lawley**

My name is Joanne Lawley and I joined MPS in October 2011 as Christine's Personal Assistant, replacing Saskia Santos who left the company in September 2011. I come from a mainly legal background with some experience within patient advice and public involvement in the NHS. I recently moved to London from Derbyshire in the summer of 2010 and am still finding my feet in an area much larger than I'm used to! However, the transition has been made much easier since joining the MPS Society. I have been made to feel very welcome by all at MPS and am thoroughly enjoying working with all staff and members, feeling inspired everyday by the dedication of those involved.

Outside of MPS, my husband and I enjoy attending comedy events and getting to know London; however, many weekends are spent travelling home to visit family and friends.

My role with the MPS Society is extremely busy but I appreciate and enjoy every moment, working in such a rewarding yet challenging environment is very fulfilling and I look forward to helping in any way I can.
Joanne Lawley j.lawley@mpssociety.co.uk

Farewell to **Lindsey Wingate**

Hello everyone, this is a sad and missing-you-already kind of a message from me. I have been very fortunate to have worked with the MPS Society along with a cracking and loving Team here in Amersham and I shall miss so many aspects that a lump is in my throat as I write. Thank you to Christine, Sophie, Gina, Antonia, Fiona, Jo, Joanne, Steve, Sue, Rebecca and although they have left Saskia and Jolanta too.

I must say huge Thank you to all the beautiful families I have met and worked with since I began to work for the MPS Society in 2010. Although the circumstances have not always been the best we have always managed to find our way through the challenges and crisis' together with trust and communication.

Thank you to all the Medical Team members UK wide, who I have met, worked with and consulted with. Your support has been welcoming and your love and experiential knowledge for the families you work with is affirming.

I will keep the memories close to my heart…..

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**

WHAT'S ON!

CONFERENCE EVENTS

All Ireland MPS and Fabry Conference: 11 - 13 May 2012

MPS International Symposium, The Netherlands: 28 June - 1 July 2012

SPECIAL EVENTS

Lancashire Family Day: 14 April 2012, Camelot Theme Park

Chessington Family Day: 10 June 2012, funded by Barclays

Childhood Wood Remembrance Day: 15 July 2012

Childhood Wood Planting Day: 21 October 2012

MPS Sibling Break: Summer 2012

MPS REGIONAL CLINICS

Manchester BMT clinic: 4 May, 25 May,13 July, 20 July, 5 October, 19 October

Northern Ireland MPS clinic: 10 May

Newcastle MPS clinic: 12 June

Birmingham MPS clinic: 22 June, 23 November

Adult Birmingham Fabry clinic: 8 May, 18 September, 16 October

12th International Symposium on MPS and related diseases

Dear Friends and Colleagues

We have great pleasure in inviting you all to the 12th International Symposium on MPS and related diseases, which will be held in Noordwijkerhout, the Netherlands, from 28 June to 1 July 2012.

By bringing patients, parents and families together with professionals, the symposium will be able to share information on all aspects of MPS and related disorders. The overall objective is to advance the quality of care and treatment. As well as musculoskeletal disease and MPS, the brain and MPS, and new approaches to treatment, the main topics of the symposium will be pricing and reimbursement. All will be covered in joint sessions attended by doctors, scientists, patients and patients' families. Separately, doctors and scientists will also attend more detailed sessions on the CNS, bone disease and novel approaches to treatment.

Families will not only have opportunities to meet peers from other countries, but will be able to attend sessions on optimizing care, dealing with clinical issues and surgery in MPS, and 'living fully with MPS'.

The symposium will be held at the four-star NH Leeuwenhorst conference centre in Noordwijkerhout, which is approximately 20 minutes from Amsterdam Schiphol airport and 30 minutes from the city of Amsterdam.

Noordwijkerhout lies very near to a coast with long sandy beaches, and various major cities and sites of interest are within easy reach. Special activities will be organised for young patients and their siblings, who will be accompanied by trained volunteers. On behalf of the organising committee, we look forward to welcoming you to Noordwijkerhout next June. *Frits Wijburg, Ans van der Ploeg, Hanka Meutgeert*

Further information can be found on the MPS website **www.mpssociety.co.uk** or by visiting **www.mps2012.eu**

**It should be noted that any families booking at this stage will not be eligible for funding and will have to pay the full cost of attending the conference and accommodation.*

New members

Megan and Ned Stringer have recently been in contact with the Society. Jessica has a diagnosis of Hurler disease. Jessica is two years old. Her brother Ethan does not have Hurler disease. The family live in the North West.

Paul Angelides and Lindsey Pascoe have recently been in contact with the Society. Beau has a diagnosis of Sanfilippo disease. Beau is one year old and the family live in the Midlands.

Miss Matthewson and Mr Clark have recently been in contact with the Society. Dominic has a diagnosis of Hurler-Scheie disease. Dominic is 4 years old and the family live in the North East.

Mrs Barnes has recently been in contact with the Society. Her daughter Jade has a diagnosis of Fabry disease. Jade is 12 years old. The family live in the Yorkshire area.

Ms Fanneran Burley has recently been in contact with the Society. Ethan has a diagnosis of Maroteaux Lamy disease. Ethan is two years old. The family live in Kent.

Ms Avery has recently been in contact with the Society. Joanne has a diagnosis of Fabry disease. The family live in the Sussex area.

Adele Jones has recently been in contact with the Society. Josh has a diagnosis of MPS I Hurler Scheie disease. Josh is 12 years old. The family live in Wales.

Mr and Mrs Ahmed have recently been in contact with the Society. Their daughters Suhila and Sara have diagnoses of Morquio disease. Suhila is 14 and Sara is 7 years old. The family live in London.

Births

Congratulations to Heather Eggleton and family on the birth of baby Jake Martin Baker Eggleton-Price, born on 6 December 2011. Heather's sister Kim suffered from MPS III Sanfilippo.

Congratulations to the family of Pam Hughes. Grace Erin Hardiman was born to Louise and Rob on 10 August 2011, much loved by her big brother Thomas. Grace and Thomas are pictured right. Louise's brother Daniel suffered from Hunter disease.

Celebrating Georgia's 16th birthday

It was Georgia's 16th birthday in July - a very special milestone indeed & we celebrated by having a big party with family and friends, at our local community centre. It was a hot, sunny day and it went perfectly.

Lots of friends helped out on the day, so fortunately for me, there was no fuss or hassle! At the party, the children enjoyed a bouncy castle, nail art, face painting and a chocolate fountain, which went down a treat!

Georgia had her face painted, she sat still for about one minute! As you can imagine, the painting was done very quickly! We have wonderful memories and photos of a very special day.

We got Georgia a new puppy for her birthday - obviously, not a decision that was taken lightly, but after a lot of deliberation, we got a gorgeous 'springador' (springer spaniel X Labrador) & we named her 'Belle', as she is really beautiful! We weren't sure how she would be with Georgia, but our concerns were unfounded, as they are inseparable! She is highly intelligent, but doesn't bat an eyelid as Georgia is pulling her ears, or poking her eyes! Not maliciously of course, but anything in close proximity to Georgia needs to be tough! Maybe one day, Belle will wise up to it, but for now, she just sits there and takes it!

Lousie Lewis, Georgia's mother

Since this article was written, Georgia very sadly passed away on 19 February 2012 aged 16 years.

Deaths

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

Lewis Browning who suffered from Hurler disease and passed away on 1 September 2011 aged 15 years.

Lewis Cato who suffered from Hurler disease and passed away on 1 September 2011 aged 1 year.

Kim van Golen who suffered from MLD and passed away on 8 November 2011 aged 19 years.

Daniel Singh who suffered from Hunter disease and passed away on 16 December 2011 aged 20 years.

Colin Arrowsmith who suffered from Hunter disease and passed away on 8 February 2012 aged 31 years.

Georgia Lewis who suffered from Sanfilippo disease and passed away on 19 February 2012 aged 16 years.

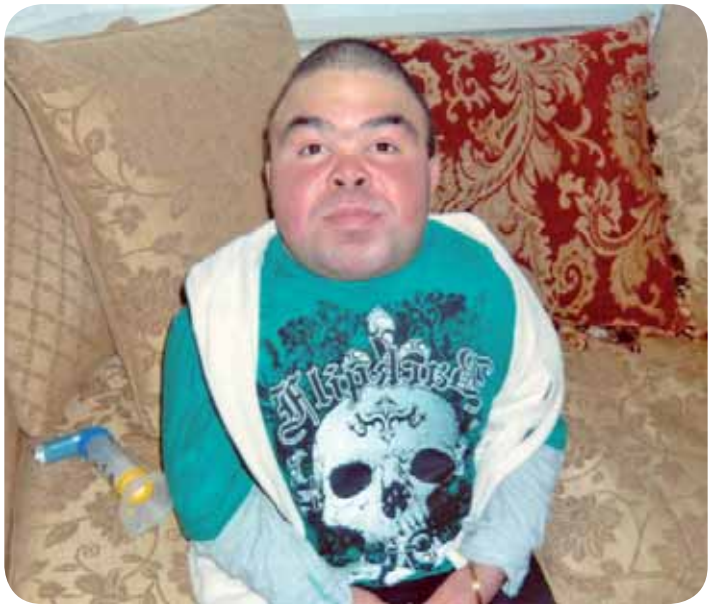
Dean Doherty who suffered from Sanfilippo disease and passed away on 25 February 2012 aged 12 years.

In memory Daniel Singh

From the moment Daniel was born he was the joy of our life. He was the fun, stars and the moon all wrapped up in one perfect little boy. The sun because of his beautiful face and a smile that would melt your heart, the stars because of the twinkle in his eyes when he raised his eyebrows to flirt with the ladies and the moon because he would rather stay awake and look at it than go to sleep.

Daniel touched so many peoples' lives and he lived his short life to the full enjoying football, swimming, horseriding, bowling, martial arts, right up to his final week. Everyone would say if Daniel tripped you up or gave you a kick in the shins you were his friend and he liked you. He had the most wonderful sense of humour. Our lives have been blessed for the past 20 years and apart from one obvious thing we wouldn't have changed a bit of it. A light has gone out in our lives that can never come back but our hearts are filled with the wonderful memories and love he has left us. God bless our darling boy and keep smiling down on us.

Daniel had some favourite TV shows that he would watch over and over again, one especially was the 'Darling Buds of May'. There is a poem in the programme that we used to say to him that he loved...



'Shall I compare thee to a summer's day
Thou art more lovely and more temperate
Rough winds do shake the darling buds of May
And summer's lease hath all too short a date.'
Sandra and Rash Singh

CONGRATULATIONS TO THE GAUCHER ASSOCIATION

On 5 November 2012 the UK Gaucher Association celebrated their 20th Anniversary in style aboard the 'Dixie Queen'. The MPS Society and the Gaucher Association work very closely together under the umbrella of the 'UK LSD Patient Association Collaborative' and it was an immense honour to be invited to join such distinguished guests on this special occasion. We boarded the 'Dixie Queen' by the Tower of London and sailed down the Thames to the Thames Barrier. Apart from the wonderful hospitality and dinner on board the boat the highlight had to be Tower Bridge opening for us to sail under with the odd firework lighting the sky from the bank of the Thames. **Christine Lavery**

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.

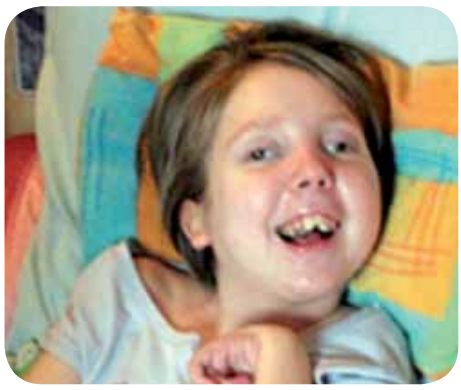
The Advocacy Support Service

In the last edition we introduced you to the Advocacy Support Team, detailing who we are, what we do, our key aims and objectives as well as highlighting some of the areas of support that we provide.

The work of the Society can vary widely and can take us anywhere within the UK. Since the last magazine we have supported over 100 families, some were short term pieces some are still on-going today. Our main request for support are in the areas of housing, education and benefits but, we are also seeing a rise in accessing social care resources and, are being required to make a number of referrals and provide information to support individual applications, due to cut backs and withdrawal of resources.

To help illustrate the types of support that the Advocacy Team has provided to its members, we have detailed below some examples of work undertaken, highlighting the involvement of the team and where possible the outcome.

Kim Van Golen (MLD)



Kim lived in her own home receiving a 24 hour support package managed by her mother and local supporting agencies. Our support for Kim and her family has involved liaising with the local social services and health providers in order to monitor provision and to ensure that those providers were managing the package effectively.

Over the years Kim's package of care had been overlooked by the authorities and as such there were a number of issues to be raised, for example staff training and CRB checks etc.

I supported Kim's mother to engage with the appropriate workers and supported her to build confidence in dealing with Kim's care and the processes and procedures associated with this. A number of areas had been neglected and over time we raised these and supported Kim's mum in getting them addressed for example accessing the garden/ going outside, occupational therapy involvement, fire risk assessments and financial management.

I also successfully applied for charitable funding so that Kim's bedroom could be refurnished and redecorated.

Sadly Kim passed away in November, I have provided bereavement support and practical advice following Kim's death e.g.: benefits advice and I attended the funeral.

Steve Cotterell
steve.cotterell@mpssociety.co.uk

Get in touch...
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0845 389 9901
or email
advocacy@mpssociety.co.uk

Isabel Annakin (MLD)

Isabel's grandmother first made contact with the Society in September this year, their initial enquiry was asking for information about the specialist centres. They had in the past had a bad experience at one of the Specialist centres and wanted to see if they could be seen elsewhere. As new members we arranged to make a home visit and here we discussed the options available to them. I agreed to look into how to make the necessary arrangements for a referral and to look at how to organise hospital transport. Whilst there the family expressed concerns around housing.

In following up this visit we contacted the Specialist centre and it was arranged that one of the Doctors would meet the family at one of their outreach clinics and we arranged hospital transport. We also attended Isabel's annual review and were able to support the family to speak openly about their concerns. We also wrote to their local authority asking that they address the housing issue as a matter of urgency. We are in regular contact with the family, their OT, social worker and care provider as well as the housing office and we are now working together to resolve the issues. At the review meeting education was also raised as an area of concern and I will now be supporting the family to access appropriate education for Isabel.

Steve Cotterell

Zara Watson (MPS III)

Zara and her family have been through many ordeals over the years. Our most recent work with the family has been supporting them through a safeguarding matter for Zara. In 2009 Zara had been subjected to very poor treatment at a learning disability and mental health facility in their local area and had been given an over prescription of a behaviour modifying drug. The family with support from the MPS Society have been pursuing a complaint against the local health trust for over two years now. We are currently still pursuing this through the safeguarding team locally, and hope to have a resolution soon.

We are also supporting the family with a complaint against another hospital following poor practice and procedures in a clinical setting. In addition to this we have been supporting Zara and her family to access an appropriate care package and to develop effective services locally. We have liaised and met with her local professionals on a number of occasions and have assisted in the assessment process. During this time Zara has been admitted to hospital on a number of occasions and we have been involved in communicating with professionals and assisting in the coordination of care provision. When possible we have visited Zara in hospital and have supported the family to raise their concerns about the care that Zara has received. Zara is now home and I am calling an urgent case review for Zara so that we can set up a package of care to meet her needs and to resolve local problems that have been experienced in the past, this is to include direct access to a hospital ward, training for GP services and lines of communication with the local hospital. **Steve Cotterell**

Luke & Olivia Vickery (MPS IV)

We were contacted for support following the family's application for a Special Educational Needs Assessment for their younger child Luke who suffers with MPS IVA Morquio disease being refused.

We encouraged the family to appeal this decision and liaised with Luke's SENCO to support the family in challenging this. Both the Society and the SENCO sent letters to the Local Authority supporting the family's views. As a result the SEN Panel gathered again and looked into all information provided. The SEN Panel decided to put Luke through the SEN assessment process. During the assessment process we provided the SEN Panel with information about the condition and its effects on learning. Eventually the Local Authority decided to issue Luke with a statement.

After some time the family applied for a SEN assessment for their older child, Olivia who also suffers with MPS IVA Morquio disease. As in the first case the application was declined. Again we encouraged the family to formally write asking them to reconsider and we also wrote advocating this. Unfortunately the Local Authority refused the request to reconsider their decision. The only option given to the family was to appeal the Local authority's decision at a Tribunal.

The Society supported the family to build up a case to present at tribunal and the Society also attended the hearing, giving information about the condition and its effects on Olivia's learning. The Tribunal agreed that an assessment should take place but could not guarantee that a statement would be issued. We provided on-going support to the family through this process and their older child was eventually awarded a SEN statement, based on her needs.

Rebecca Brandon
r.brandon@mpssociety.co.uk

Dylan Tonge (Mannosidosis)

The Society received a call from a family whose child has Mannosidosis. Dylan had a school placement in a mainstream school with a centre for the Deaf. He was spending most of his time being taught in the centre for the Deaf and part of the time with his mainstream class. His parents with support of the school applied for a statement of special educational needs as their son required 1:1 support during his core subjects.

Rebecca Brandon

The Local Authority drafted a statement, however, it did not cover all Dylan's needs and how his condition would impact on his learning. They asked if we could support them with this by providing specific information on his condition and the needs he would likely have in school.

Following this submission a meeting was called to review the proposed statement. The Society attended the meeting to give support and advice regarding the effects of the disease and to discuss the report and the specific concerns of the parents. The Society was then asked to attend a further meeting at the school to discuss the needs of the child; the School agreed that he required more 1:1 supervision on focused activities. The Special Educational Needs Officer was going back to amend the statement to cover all the areas discussed. This was agreed and a new statement issued. At a recent review meeting his statement was maintained and his speech and language therapy was reinstated after a period of absence.

Dylan's parents greatly appreciate the help given by the MPS Society, particularly Rebecca Brandon's support and advice which has been invaluable.

Roma Drayne (MPS IV)

One of our members with MPS IVA approached the All Ireland Advocacy and Support Service for support in communicating her need for assistive technologies to her Occupational Therapist. The individual was unable to function independently in their home due to difficulties in opening/closing doors and controlling lighting, curtains etc. The family had hoped to apply for a disabled facilities grant (DFG) to have assistive technologies such as automatic doors and remote control systems for lighting etc. installed.

Following discussion with their OT the family were told that these adaptations could not be provided because they are not included in the OT guidelines.

A report was provided to the OT outlining the potential value of having assistive technologies installed. Due to lack of progress the family made a formal complaint in relation to the service provided by Occupational Therapy services.

This complaint was formally addressed at a meeting held at the family home. The family were supported by the MPS Society at this meeting. Following this meeting it was agreed that the individual's OT could recommend that automatic doors and an intercom system be provided through a DFG. Remote controls for lighting etc would be provided directly from occupational therapy services.

Several months on the family are continuing to struggle to have their needs understood by the professionals involved. Some assistive technologies have been put in place but additional adaptations are required to facilitate independence. There have been several meetings with their Occupational Therapist and the Grants Officer involved in their application. The MPS Society is in continuing contact with the family and are supporting them in writing to the Minister for Health to highlight their needs.

Alison Wilson
a.wilson@mpssociety.co.uk

Support requests

The Society is keen to hear from anyone who requires any help or support. This may be a letter of support, attendance at a meeting or even just a friendly chat with someone at the other end of the telephone.

Farewell to Lindsey

As some of you will already know the Society has had to say a very sad farewell to Lindsey Wingate who for personal reasons decided to leave the MPS Society. Lindsey has written her own personal farewell message but on behalf of the MPS Society and trustees I would like to thank her for all her dedication and hard work, especially involving the transition project and wish her well for the future.

Future articles / information

Please look out for the next magazine where we will give you an insight into what it is like to be an Advocacy Officer at the Society, with our article "A day in the life of an Advocacy Support Officer".

In addition to this, if anyone has anything specific they would like to see shared in this new chapter of the magazine please do not hesitate to put forward your request directly to me on the details below.

Sophie Thomas
s.thomas@mpssociety.co.uk

Get in touch...

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

All Ireland Advocacy Support

The All Ireland Advocacy and 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 the work we do in Ireland and what we hope to do in the future.

N. Ireland MPS Clinic

The last Northern Ireland Winter MPS Clinic was held on Friday 9th December 2011.

This was one of our busiest Northern Ireland Clinics so far! The medical team - Dr Fiona Stewart, Dr Simon Jones, Mrs Jean Mercer, Mrs Janice Scott and Dr Deidre Donnelly - had appointments with 12 of our members and I was able to catch up with all of the families who attended. As usual the clinic proved to be the perfect place to discuss family support. Since the clinic, I have been travelling around Northern Ireland meeting families in their homes to discuss their needs.

We were delighted that Jean Mercer from the Willink in Manchester was able to attend this clinic. Jean made the trip over to Northern Ireland to catch-up with some of the MPS I patients who she has been involved with – and I'm sure I can speak for all the families when I say that they were delighted to see her. Thank you Jean, we hope to see you over in Northern Ireland again soon.

The next NI MPS Clinic will be on 11th May 2012 (why don't those of you who are attending make a weekend out of it and head over to the Hilton Hotel (Templepatrick) for the launch of the All Ireland MPS Conference that evening!)

Fabry Clinic

The last Joint Cardiac and Genetics Fabry clinic was held on 16th December 2011. It was great to catch up with some familiar faces and find out how the last year has been for them. As a newly established clinic we are keen to receive any feedback you have. The next joint Cardiac and Genetics clinic is on 20 April 2012.

The Northern Ireland Rare Disease Partnership

As a representative of the MPS Society I have been attending some initial meetings of the Northern Ireland Rare Disease Partnership (NIRDP). This is a group of individuals, and representatives of organisations, with an interest in Rare Disease. The group aims to work together to advocate for individuals with rare diseases in the following areas:

- o Education
- o Access to Specialists
- o Lobbying
- o Co-ordination of Services
- o Support for families
- o Organisational Development

The Northern Ireland Rare Disease Partnership was officially launched on Rare Disease Day (29th February 2012) at an event that took place in the Pavilion on Stormont Estate in Belfast. Some of our members were involved in this event. I hope to bring you a report on the NIRDP Launch in our next magazine.

I will be continuing to attend NIRDP meetings to ensure that individuals with MPS are given a voice in this forum.

S. Ireland MPS I Clinic

On 18 November 2012 I attended the MPS I specialist clinic at Our Lady's Children's hospital in Dublin. At this clinic I was able to meet with 12 individuals with MPS I and their families. Some of these families had previously made contact with our service, so it was lovely to finally be able to match faces to the voices on the other end of the phone. I was delighted to be made to feel so welcome at this clinic and look forward to attending the next clinic in 2012.

Many of the families attending this clinic brought with them some significant support needs and I have been working closely with those families to meet their needs and give them the support they require. I hope to bring you testimonials from some of the families I have been working with in the next issue to the magazine.

Working with the Traveller community

Following the development of the Hurler Awareness DVD in 2011, I have been contacted by representatives from Traveller groups across Ireland to discuss showing the DVD in their areas.

It was felt by some Traveller Groups that they would require some education about MPS and related conditions before showing the DVD to families in their areas.

All Ireland Advocacy Support...

So, in the coming months I will be delivering some education sessions to the staff of Community Traveller Groups – these sessions will provide a 'crash course' on the everything from genetics to family support.

The first education session was held in February 2012. I am really looking forward to these sessions and hope that they will help to increase awareness of MPS and the needs of individuals affected by MPS in the Traveller Community.

Adult Services for Metabolic Disease

Towards the end of 2011 I attended a meeting in Temple Street Metabolic Unit with Professor Eileen Treacy, some members of the Irish MPS Society, the parent of a child with PKU and the founder of Fabry.ie to discuss the development of a service for adults with metabolic disease in Ireland. This service is desperately needed and we hope that adding a the patient voice to the voices of the professionals will help to move things forward.

Those who attended the meeting have taken on the work previously started by the 'Inherited Metabolic Disease Taskforce' and will be taking steps to highlight the need for the development of an adequately staff Adult Metabolic Service.

To contact Alison email a.wilson@mpssociety.co.uk

Sarah's update...

Sarah was featured in the previous edition of the MPS Magazine. Sarah has written to update us on what she's been up to since then. Sarah is 21 and has Fabry...

Well what's happened since I last wrote? I've finished another year of my Open University degree so I'm now onto the next one. I've got used to the amount of work expected of me now so it's getting a little easier.

Christmas time was a little tricky, only had one week with no Open University work to do, so with extra Christmas hours at work and coursework to do it was a little tricky, didn't want to overwork myself and make me ill.

You can never tell when a bad Fabry day is going to hit. Usually the cold weather means the onset of some kind of bug or another, working in a shop, you get the lovely customers sharing all their germs with you, and I always seem to catch everything, luckily this year has been ok and I've not gone down with too many.

I also ventured to pastures new in January and visited New York with my brother. Going on holiday can also be tricky, I can't plan too many things before I go away as I cannot tell when a Fabry attack is going to hit, having an attack would have meant that I wouldn't have been able to go out until it passes, painful hands and feet aren't a good companion to walking around in the cold. New York was amazing, I have never been before so it was a good experience. January isn't the best time for weather though, it was freezing and we had a snowstorm, it was pretty but made it very cold.

I do find however that I am much better going away at this time of year though as the cold doesn't seem to affect me as much as the heat. I'm more likely to have an attack if I am in a hot place and therefore need to be more careful, but its definitely worth going away and not feeling like I have to be stuck at home just because I cannot tell if I'm going to be ill. If I am I deal with it but if you don't go you might regret not seeing a new place and will never know what you are missing.

Anyway, here's to a few more good months ahead and another exam in the summer. **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**

Fun at St Fagans

This is a photograph of me when I was awarded an award for Promoting Teamwork at St Fagans with a good friend of mine, Sam Williams who is also deaf, the day was actually on my birthday which was a bit of a surprise.

Megan Rennoldson has Mannosidosis. Here she tells us all about her Teamwork Award and the work she does to raise funds for charity...

My father came along and with two friends of mine watched me be given this Award.

I am still doing local Coffee Mornings in Monmouth trying to raise money for Action on Hearing Loss and other charities.

Slimming World Award
I started my weight at 12st 9lb and I have not stopped going every Tuesday.

The reason why I wanted to go to slimming world is to do something about my weight which I have done now what a acheivement for me. I am now 9st 9lb which is great!
Megan Rennoldson

Well done James!

James Fair, who has MPS II Hunter disease, started horse riding at etal RDA group on 28th February 2010.

This was suggested by James' paediatrician as he was getting no PE at school and becoming very frustrated into the bargain. James seemed to enjoy the first few sessions and gained in confidence each time he rode, we were asked if he would like to enter a competition to qualify for the national championships at Hartpury in Gloucestershire.

They went along to the qualifiers in May and low and behold after only 3 months of riding, James qualified and went on to win a first for the Countryside Challenge and 4th in the dressage competion.

The family set off in July for Hartpury and again James did really well, coming 4th in the dressage, 2nd in the Countryside Challenge, gaining a distinction in horse care and knowledge, first in the combined class (dressage and countryside challenge) and a 5th in the regional team event. However, his best result was winning class champion for combined class with both adults and juniors put together. James won a rather nice trophy as well as another rosette. This was quite a win for a child who had only been on a horse for five months.

Such a proud moment for us all including his brother William who was kept busy with the camera. Special thanks to "mac" and "patsy" and all the volunteers at etal rda.
The Fair Family

Chiara's story

Chiara has Hurler disease. She has recently undergone a Bone Marrow Transplant. This is the story of the family's experience receiving the diagnosis and their hopes following BMT.

After a terrible pregnancy on 20 July 2010 Chiara Petersen Fox was born by emergency section. She looked perfect in every way. On her second day of life she had her newborn hearing test and unfortunately failed this. We were told not to worry that she could still have some fluid in her ears and she would be re-checked in about 2 weeks.

Over the next few weeks she fed, slept and gained weight then gradually everything seemed to deteriorate. She struggled to feed, she had extremely noisy breathing and she made a strange snorting noise. We had returned to have her hearing test repeated and unfortunately this was incomplete and had to be rechecked but already I sensed she was not hearing.

By September she had failure to thrive, struggled with milk feeds, had poor head control and in general I knew there was something wrong. She was admitted to hospital in October and initially she was thought to have Retrognathia, although I was not convinced of this after researching it on the internet.

She was transferred to Yorkhill Hospital from our local hospital and it was there she was diagnosed with Laryngomalacia (in simple terms a floppy windpipe). She underwent surgery for this the following day and we thought this was going to be the answer to all our problems. She was discharged home a week later but unfortunately she had developed a terrible cough.

After only a few days at home Chiara was readmitted to Yorkhill Hospital. She was observed on the ward for a few days then she underwent a second lot of surgery for her Laryngomalacia and also had an ABR carried out to determine hearing loss. She didn't recover very well from this surgery and unfortunately after only a couple of days on the ward was admitted to Intensive care through the night for respiratory distress. I felt so helpless as I stood and watched her struggle for breath.

The results from several tests carried out confirmed she had Whooping Cough. She remained in hospital for a period of weeks and then discharged home. She looked much better and was feeding slightly better. She was fitted with hearing aids for bilateral moderate to severe hearing loss. She was discharged home with an Apnoea Monitor as she was suffering apnoeic episodes while sleeping.

Unfortunately we had discovered a lump on Chiara's spine around this time and it was then I knew she must have a more serious condition. At this point she had been diagnosed with Laryngomalacia, Reflux, Milk Intolerance, Scoliosis, Deafness and Respiratory problems. She also had very poor head control and in fact her whole muscle control was poor for her age.

In January 2011 Chiara developed continuous secretions from her nose, nothing helped, even after two courses of antibiotics. By February we had been referred to an Orthopaedic Specialist and Genetics to find out if Chiara had a syndrome of some sort. She continued to deteriorate in front of our eyes and on 4th April 2011 we attended A&E with Chiara as she had turned blue. She was checked by several doctors who couldn't understand what was wrong, she was struggling to breathe and by midnight the retrieval team from Yorkhill was telephoned to come and take her through. It was the worst experience ever, we watched her tiny body placed on a huge stretcher and her taken away in an ambulance with us following on.

She remained in ITU after being intubated and ventilated. It was then after our distressing and upsetting experience I knew I had to find out what was underlying with my girl.

She had MRI scans, Neurology assessments etc but we still received no answers. She was suffering extreme apnoeic episodes and a sleep study revealed she was desaturating hundreds of times a night. The following day Chiara had an artificial airway inserted to help with her airway and right away this made a huge difference. A sleep study that evening showed although the NPA was helping she was still struggling to breathe while she slept. The following day she was started on a BIPAP machine at night which would circulate positive pressure through a mask while she slept. The result of the sleep study showed this worked and she had a great night sleep.

Although this was a huge step forward we still had other issues to be addressed. Another Doctor from the Metabolic Team was now to be involved. He carried out several tests including urine and x-rays, the results of which would take around two weeks.

On 29th April 2011 we were given the devastating news that Chiara was found to have a Storage Disorder. Three days later we were told it looked likely Chiara had MPS I (H) Hurlers. Devastation doesn't even come close to how we felt. I cried for what seemed like forever. We were told that once it was confirmed then our options would be discussed with us.

Around a week later we had the horrid ordeal of listening to our wee girl being confirmed with MPS I(H). We were offered Enzyme Replacement Therapy (ERT) and a Bone Marrow Transplant (BMT). Only a few weeks later Chiara received her first dose of ERT. By June we were leaving the hospital with a little girl who had now gained loads of weight and had better head control and although at this point was still unable to sit unaided was making huge steps in advancement.

In July we returned to hospital where Chiara was now sitting alone,trying to crawl and on her birthday had her artificial airway (NPA) removed as her breathing had improved greatly. Her secretions also had disappeared. On 17th August Chiara commenced Chemotherapy for BMT on 24th August 2011. She coped fantastically with the Chemo, she went through the whole transplant process with no NG tube and took all medications orally and was fed a normal diet. She developed no mucositis and did not really have much breakdown of her skin.

She had her life changing BMT on 24th August 2011 which was her brother's birthday. She received a cord blood which was 6/6 match. She recovered really really well and amazed the doctors.

This wee girl who had initially shown to be high risk because of her airway problems was thriving and within weeks of transplant she had a sleep study carried out which revealed her to have normal breathing off her BIPAP machine and therefore she no longer required BIPAP ventilation at night. By the time we were ready to leave hospital it had been noted that her hearing had improved, she was now pulling herself up and standing. It was a miracle.

We left the hospital within 8 weeks of entering it to start Chemotherapy. A follow up hearing test confirmed that Chiara's hearing had changed from moderate/severe hearing loss to mild hearing loss and at present does not require her hearing aids. It does state in a lot of articles relating to MPS I(H) that hearing does not improve because of the nerve damage but in Chiara's case this has been proved wrong.

She is now slightly overweight, she walks around the furniture, she has started talking, she has no signs now of developmental delay and the huge thing is she can hear. Next year she will receive her spinal correction surgery.

As I write this article we have 8 days to go till we reach day 100 of transplant. I honestly can't believe how much our whole life has turned around since receiving Chiara's diagnosis of Hurlers. She is so healthy now and does everything a normal healthy girl of her age can do. She continues to be the happy, charismatic, loveable girl that melts the hearts of everyone she comes into contact with.

"She is our adorable, miracle little Princess. She has been an inspiration to everyone who hears of her and all she has had to endure in her short life so far."

www.chiarafox.blogspot.com

Manchester MPS I BMT Under 6's Clinic

14 October 2011

The last clinic in 2011 for children under 6 years old took place on 14th October. As usual I jumped on a train at Watford Junction and two hours later I arrived in Manchester. Quickly I hopped in the taxi and soon after I was saying 'Hello' to Shantelle, her mum and sister Shirley. Shantelle also came on a train so we exchanged pros and cons of this means of transport.

Next one to arrive was Ethan who brought his parents and nan with him. Ethan hasn't changed a bit, except for few extra inches in his height. He still tried to climb everything - the higher the better.

After Ethan came Holly with her mum. Holly had her uniform on and she was asking when she was going back to school. As you can guess Holly absolutely loves going to school!

Jamie flew over from Ireland with his mum. He straight away set about building high towers and destroyed each one with the cheeky grin on his face.

I also said 'Hello' to Jessica who came with her parents and baby brother Ethan. Jessica was very interested in everything around her and all the time on the move. Smiling at everybody but not when I tried to take a photo. Maybe next time. **Jolanta Turz**

Photos this page clockwise from top left: MPS I BMT Under 6'S Clinic - Chantelle Reilly, Holly Campbell, Ethan Greening, Jessica Stringer, Jamie Topkul.

Manchester MPS I BMT Over 6's Clinic

21 October 2011
A week later after seeing the little ones I came to Manchester for the very last clinic this year.

The first to arrive was David with his parents. David is a proper rock star now. He plays acoustic guitar and recently got an electric one as well. Dad was showing videos of David playing and I will not be surprised to see him on stage in a few year's time.

After David arrived Cody also with both parents. Cody was very excited because she was going to Lapland in December. She was dancing around and struck a pose for the photo with her pal Millie. The monkey called Green Chicken (?!) stayed at home this time.

Then Melissa turned up with her mum. Melissa also had exciting news to share, she was going to Disney World Florida in couple of days. All the family was packed and ready to go. Although Melissa looked very serious in her new purple uniform she could not hide the excitement.

Then as usual, double trouble arrived - Rachel and Charlie. Charlie was ushered quickly for his weight and height and to see consultants and I stayed and had a chat with Rachel and her parents. They were also very excited because they were going to Disney World Florida as well!

I would like to say a big thank you to the Manchester Team, for both clinics, for all your hard work and for making the families and I so welcome. **Jolanta Turz**

Photos this page, left to right: MPS I BMT Over 6's Clinic - Cody Taylor, Melissa McKie, Rachel Rothwell and her brother John

Birmingham Children's MPS Clinic

18 November 2011
Well it was another early start to get to the Children's Clinic. It was lovely to see that Cat has come back from maternity leave and she was trying to catch up with what was happening with everyone.

First on the starting blocks was Lisa Marie looking as always, very stylish. Anya looked lovely and seemed to be doing well.

Roman and Xena were into everything and ran off with the camera and decided to take some pictures of their own, sorry guys we had to use ours, yours were… blurred.

Muhammad arrived with his mum, dad and big brother. I think too much going on for his liking. Asif chatted away while he was waiting for his appointment, and seemed happy enough not to be at school.

Fahim was a little star waiting patiently with his mum until he was seen.

I think Sohail and Humza must like the trip to Birmingham as they were tucking into lots of goodies!

Well, soon it was time for Jolanta and I to make our way back to Buckinghamshire. It was Jolanta's last clinic before she leaves the MPS Society to go to pastures new, well Prague, to be precise.

She sends all her best wishes to the staff and patients and leaves you all in the capable hands of the rest of the advocacy team. Thank you to the team for looking after us.

Rebecca Brandon
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Photos this page clockwise from top left: Birmingham Children's Clinic - Anya Bhatti, Lisa Marie Barr, Roman Riaz and Xena Begum, Muhammed Safeer with his big brother, Asif Muhammed, Fahim Hussain

Birmingham Adult Fabry Clinic

17 November 2011
It doesn't seem five minutes since I went to the last Adult Fabry Clinic.

This time we were in the new Queen Elizabeth Hospital. It certainly is a very impressive building, I had a bit of trouble trying to get to the right car park, sat navs are all very well but they don't know if someone has kindly put in No Entry signs!

Liz kindly guided me to the clinic area and I met with the patients and the genetic team who were visiting the clinic for the day.

There were some old faces and a couple of new people who I had not had the pleasure of meeting before

The new location is very spacious and airy and I think everyone agreed that the new hospital is not a bad place to go.

Thank you to Dr Hiwot and the team for their hospitality.

Rebecca Brandon
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GOSH MPS III Clinic

6 December 2011
This clinic was a little quieter than usual due to cancellations however those who did attend definitely kept their parents and the team at GOSH on their toes…. doors are meant to be opened! Niamh, Victoria and the team made us all very welcome, so we would like to say thank you for your hard work and we'll look forward to the next one.

Steve Cotterell
steve.cotterell@mpssociety.co.uk

Photos this page clockwise top row - left to right: Birmingham Children's Clinic - Humza Mumtaz, Sohail Mumtaz, bottom row - left to right: GOSH MPS III Clinic - Bobby Gill, Lily Brooker.

MPS I BMT Over 6's Clinic

20 January 2012
The Manchester clinic was the first one that I had attended in 2012. It was raining rather than snowing which is the main thing.

The train was on time and as usual I had the third degree from Isaac about where I had come from and which platform I arrived at and how I was getting back. He was very proud of his school uniform and wanted to get back post haste.

Rubina looked lovely and knew the drill and sat and had her picture taken.

Mikko made his presence felt by coming into the department with a flying dive along the floor, (he was fine).

Thomas had a chat about his Christmas presents and his holiday, followed up by Leighton who already has his life planned out for going to college!

Luke arrived with his mum, dad and brother. Luke decided to build a very large tower of lego, but I suspect it was more fun to knock it down than build it.

The parents all had the opportunity to catch up and see how all the children were getting on in school. Soon it was time to go home. Thanks to the team for making me welcome.

Rebecca Brandon
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This page, top row (l-r) Isaac Turner, Rubina Jalani, Mikko Astle.
Bottom row (l-r) Thomas Mett and his Mum, Leighton Barker, Luke and his brother Quinton.

MPS I BMT Under 6's Clinic

27 January 2012
The BMT clinic for under 6's was held at Manchester Children's Hospital. A busy morning by all accounts with families coming from far and wide to see Ed, Jean and the team.

There were opportunities for families to meet and catch up and for them to talk to me about on-going issues and I know that there is some excitement about the forthcoming Camelot family day.

On behalf of the MPS Society and all the families I would like to thank the team at the Willink for their continued support.

Steve Cotterell
steve.cotterell@mpssociety.co.uk



Photos from left to right: BMT clinic under 5's - Alicia Evans, Jake Little, Morgan Wright.

MPS Awareness Day

15 May 2012

One baby will be born every eight days in the UK with an MPS or related disease

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

Help us celebrate International MPS Awareness Day on Tuesday 15 May 2012

In 2012 we're asking all our members, Friends and supporters to do something, big or small, to mark MPS Awareness Day

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…

Find us on Facebook

Childhood Wood Planting

Children and adults who lost their loved ones to MPS and related diseases in the last year were remembered in the Childhood Wood at Sherwood Pines, Nottinghamshire on Friday 21 October 2011 as part of our annual programme of events at the Childhood Wood.

In a break with tradition the dignitaries, trustees, families and I gathered at the Sherwood Pines Forest Cafe before making their way to the Childhood Wood where they were able to see their loved one's names on the Remembrance Boards as well as explore the Childhood Wood and see some of the children's activity areas put in place with a grant from the Geoff and Fiona Squire Foundation.

After a short remembrance ceremony blue and white balloons were released followed by the planting of an oak tree sapling for each of the children and adults being remembered.

Christine Lavery c.lavery@mpssociety.co.uk

THOSE REMEMBERED

Jasmin Heap
Matthew Hodges
Jordan Lacey
Simon Lavery
Michael Paul Sean Megoran
Elijah William Moore
Harrie Matthew Moore
Helen O'Toole
Gethin Robins
Matthew Kyle Wright






Scottish Post-Christmas Blues party

The MPS Society was delighted to host a party in January to fight off those post Christmas blues. We welcomed a number of Scottish families and enjoyed some festive fun, good food and magical entertainment!

Many people can feel a little low in spirit after the Christmas festivities are over, so when we were awarded a grant to provide a get together in Edinburgh Scotland, we thought that it would be a nice idea to have something to look forward to and decided to hold a Post-Christmas Blues party on Sunday 15 January. 2012 This was well received and we had 35 people book for this event which was held in the Hilton, Edinburgh airport.

Gina and I made the early morning trip up to Scotland and although chilly, we were thankful that there was no snow.

The party started off with a three course meal, while the children were being entertained by our regular magician and balloon modeller Gary Dunn. Gary made some exceptional balloons such as spiderman, Mario, an alien, a ladybug bracelet and a helicopter. After lunch, Gary turned his hand to entertaining us with his magic show. I'm sure that all those present would agree that his act was exceptional and for me personally it was a real feel good performance which left me laughing from start to finish. The award however, for sportsmanship of the day, has to go to Kevin, who assisted Gary in the final act of the show and for which brought the most laughs. All I will say on this matter is "I'm a little teapot."

As the party was drawing to a close we had one final guest appearance from Santa, who came especially to deliver some presents that had been forgotten before he went on his summer vacation to Barbados!

The party was a great success and I would like to thank all the families who came, it was lovely to see you all and I would also like to thank the Hilton and Gary Dunn for helping to make this party a success.

Sophie Thomas
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A magical trip to Lapland

Following the much-appreciated grant of £20,000 from the Gosling Foundation, the MPS Society was able to organise an amazing trip to Lapland for families affected by MPS and related diseases, 11 - 14 December 2011.

Day 1: Sunday 11th December
We gathered at Gatwick and flew out to Kittila in the far north of Finland, a four-hour flight, where we were taken to Hotel Levitunturi to settle in with a welcome drink and then a very welcome dinner!

The hotel and facilities were excellent. During our stay families made full use of the water world which included 17 indoor and outdoor pools! There was also a health spa, with 9 saunas and massage facilities for parents to enjoy, when worn out from all the family activities. Other facilities included a children's playhouse with ball pool, toy cars and craft materials.

Day 2: Monday 12th December
After breakfast everyone enjoyed an exhilarating morning of outdoor activities including tobogganing, snowmobiling, a husky dog ride and a reindeer sleigh ride through the winter woodlands with warming log fires and hot drinks along the way. Everyone had to wrap up as warm as possible, as the temperature reached - 27°C! We couldn't believe how cold and dark it was, with only one hour of sunlight at around midday!

Once we had returned from our adventures in the snow, we were then initiated into the arctic wilderness with a magical Arctic Circle Ceremony. A traditional Sami guide enlightened everyone with tales of this traditional ceremony, and everyone was delighted to receive a gift of a Lappish bell. After supper we all gathered together again at the top of a hill for a campfire and got into the festive spirit with a rousing carol service.

Day 3: Tuesday 13th December
This was a morning of leisure and some families chose to go skiing or sledging, while others relaxed and enjoyed the hotel facilities. One family chose to take part in another husky ride, and young Cody aged 7 was thrilled to receive a prize in the "Name the Husky" competition! After lunch families were taken to Santa Park, where they visited Santa's workshop and Post Office. The children posted Santa their Christmas present lists, saw his sorting office and watched his elves making toys.

As the evening approached, there was a buzz of excitement as the children knew that it was nearly time to meet Santa Claus. Everyone gathered for a festive Christmas Gala Feast, and after the meal Santa arrived with his sleigh of gifts for everyone! The children each got to meet him and receive their present. They were so happy to have had this special experience. There was then a disco for everyone to enjoy.

Day 4: Wednesday 14th December
Everyone gathered for their last breakfast, and then had a final opportunity to make the most of all the indoor and outdoor activities on offer.

After lunch we departed the hotel for Kittila airport and caught the flight back to Gatwick, feeling exhausted from all the activities and full of memories of their wonderful trip. We had been to a magical winter wonderland and met Santa himself, and everyone received a certificate showing all the experiences they had had, to keep as a memento.

This trip was a magical experience, remembered forever by all the families who went, and we are so grateful to the Gosling Foundation for having made this possible.

The Murty Family in Lapland

Thank you for the wonderful opportunity that was our magical trip to Lapland. I must tell you of the many great activities we experienced as a family.

Our youngest daughter Nadine suffers from MPS I (Hurlers) and although she is only three years old she has already spent far too much time in hospitals, so it was good to get the chance for her to visit Santa and have some much needed fun with mum, dad and big sister Chloe who is nine.

From the moment we met Gina and Sophie from the MPS Society and all the other lucky families at Gatwick airport you just knew there was a magical feeling in the air. Also there to greet us were Rudy the reindeer and Hal the husky who would pop in to see us again in Lapland. The flight was great and a special thanks must go to the staff who led us all in some carol singing to set the mood for a wonderful few days. On arrival at Kintilla airport we were kitted out with snowsuits and snowboots which would prove to be invaluable in the freezing temperatures of the arctic circle.

The hotel was great and was well equipped with plenty going on to keep the kids occupied, from ten pin bowling for the older kids to the soft play area for the younger ones. The swimming pools and spa area was also out of this world and were a favourite of mum and dad but the kids much preferred being out in the snow.

During the trip we also got the chance to try tobogganing, snowmobiling and were taken on reindeer and husky rides. The most special part of the trip was the visit to the north pole to see Santa's workshop, post office and house, where all the elves were hard at work on the final preparations for the big day. We got to see the elves sorting letters and making toys, and we also met Mrs Claus who was out feeding Santa's reindeer. Just as we were about to leave the north pole, some of the kids saw Santa coming out of his house and we all rushed over to meet him. The kids then invited him to our gala dinner back at the hotel, and although he was very busy, he promised to come along.

Before dinner we all got wrapped up in our snowsuits and headed into the forrest for some carol singing around a log fire and then back to the hotel for our date with our special guest. Santa duly arrived with his elves and Rudy and Hal, whom we had met at the airport days earlier. Santa spent a lot of time with all the families and showered the kids, and also the adults with gifts and we then danced the night away at the party disco.

Thank you again for the chance to be part of something so special and for giving the kids some wonderful memories to cherish. The most special thing for my wife Josie and I, was the chance to speak with other parents of children who have MPS and related disorders and to share experiences and stories of living with these types of diseases. We met some really special people and have made some great friends, thanks to being invited on this trip.

Many thanks and best wishes.
Kevin Murty

Jacob's trip of a lifetime

We just wanted to say how much we enjoyed the trip to Lapland and to say thank you to the MPS Society for organising it.

Jacob and his younger brother Samuel had a great time on the Husky and Snowmobile rides and were really excited to meet Santa and his Reindeer.

It really was a once in a lifetime trip and we want to let everyone at the MPS Society know how much we appreciated the opportunity to take Jacob to such a special place and make him so happy.

Here are two photos of us. Jacob loved watching people skiing and snowboarding down the hill.
Chris & Hannah Brentnall (parents of Jacob, MPS III)

MPS Awareness Day

15 May 2012

One baby will be born every eight days in the UK with an MPS or related disease

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

Help us celebrate International MPS Awareness Day on Tuesday 15 May 2012

In 2012 we're asking all our members, Friends and supporters to do something, big or small, to mark MPS Awareness Day

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...

Family Day in Manchester

The day had been arranged specifically for the families of children that have been taking part in the Morquio study for some considerable time. The families take part in the study at different specialist centres in England and Scotland so the day gave them the opportunity to meet other families in the same situation as them.

Saturday 21st January saw Gina and I travelling up to Manchester for our long-awaited family day. The idea for the day had first come about in July 2011 and it was finally here!

The day started with the families arriving at the Copthorne Hotel Manchester for pre-lunch drinks before we sat down to a delicious carvery. A range of starters, the main course and a selection of desserts were set out for us, which gave many people the opportunity to plan their lunch around their favourite dessert! [picture 1 shows Archie, Isaac, Enola & Cora (a sibling) eating the meal & picture 2 shows Kamal eating his dinner].

After lunch, the children (Archie, Isaac, Kamal and Enola) rushed off to see what mischief they could get themselves into. It was lovely to see them playing together and it gave the families the opportunity to catch up or get to know each other.

A little later in the afternoon our taxis arrived to take us to Old Trafford football grounds, home of Manchester United Football Club! However, we had to find the children before we could make our way over. Perhaps 'hide and seek' was not the best game they could have been playing at this point…

Once at the football grounds, Isaac treated us to a rousing rendition of 'Glory Glory Man United', we were all handed our passes for the tour and our tour guide came to meet us. Unfortunately, he brought the rain with him so we waited for a few minutes for the shower to pass.

Finally, the much anticipated moment had arrived and we made our way into the stadium. We went a little way up into one of the stands where we could look out over the empty, historic site. Many pictures were taken by some of the families! [Picture 3 is a shot of the football grounds & picture 4 is a group shot of everyone stood in the stand].

Photos 1-6 clockwise from top left

We then visited the lounge where the players relax before a match along with any invited quests; the manager can only enter this room if he has been invited by the captain of the team! We were even shown the chairs that David Beckham and the then 'Posh Spice' were sat on when they first met! Archie was much more interested in 'International Honours Board' though, which detailed all of the players that have won an international cap while playing for Manchester United.

Our next stop was a room displaying all of the player's shirts, which was situated opposite the changing rooms. I think all of the children managed to find the shirts of their favourite players (as did some of the grown-ups!) and we managed to get a few pictures of them stood in front of them. [picture 5 shows Isaac in front of a shirt and picture 6 shows Kamal with his in Grandad in front of the shirts].

Onto the tunnel now where the players make their entrance onto the pitch and the children had great fun running through the tunnel as if they were going to make their grand entrance to their adoring fans. From here we were taken to the 'dug-out' area where Alex Ferguson and the substitutes sit during the game. This seemed to be the most exciting part for the children and they badgered the tour guide with many questions about who sits where. [Picture 7 is a group photo at the exit of the tunnel, picture 8 shows the children sat in the 'dug-out' including one of the siblings and picture 9 shows the children stood in front of the team's logo at the 'dug-out' including one of the siblings].

The tour ended with a visit to the museum, which included an impressive collection of trophies, information about the devastating Munich Air Disaster of 1958 and The Hall of Fame; a dedication to United Legends. [picture 10 shows Isaac with his dad in front of a cabinet of trophies and picture 11 shows Enola with some trophies].

All too soon the day seemed to come to an end and it was time for the families to start making their way home.

I enjoyed meeting the families and we sincerely hope that everyone who attended the day enjoyed themselves. Our thanks go to the staff at the Copthorne Hotel Manchester for their help in organising a lovely day.
Picture 12 shows the club's logo.

Jo Goodman
j.goodman@mpssociety.co.uk

Photos 7-12 clockwise from top left

Volunteering opportunities

Hannah's Story

I've been volunteering for the MPS Society for a few years now and I love it.

One of my brothers (Dan) and my sister (Amy) both have Sanfilippo and when I was younger I really enjoyed coming along to the MPS conferences. Our family used to need quite a few volunteers and I have really happy memories of fun volunteers who gave us all such a great time. As a volunteer now, seeing the younger MPS children and their siblings brings back happy memories of when Amy and Dan were little and needed fast runners to chase them!

It feels really good to be able to give something back now. The weekends are always such good fun. What could be more fun than a hotel full of MPS kids running around and causing chaos!

It's great to meet some amazing families and spend the day with fantastic kids in the hotel or taking them out for the day to a theme park or zoo. It's definitely hard work and challenging at times and you need to be prepared to run fast and have hands that can be in 4 places at once!

Sometimes things don't always go to plan either… like when fire alarms go off and you have to evacuate all the children out of the hotel! But it's all great and by the end of the day a good night's sleep is always guaranteed! It's satisfying too; I really enjoy getting to know the kids and their siblings over the weekend. Seeing them enjoy themselves whilst knowing that their parents are able to appreciate a bit of a break is great. It's nice to feel like you've really been a help and it's really rewarding to have a parent say to you "Wow you can come again, she's gone straight to sleep she was so worn out!"

Being trusted to look after someone else's child for the day does feel like a big responsibility, but the weekends are always so well organised. The volunteers are like a big team and all work together and help each other out. It's been good coming back year after year, catching up with all the other volunteers and seeing the families that I've got to know before.

This last year it's been really nice to volunteer on the young adult weekend too. This was in Blackpool and was quite a bit different to the other weekends I'd volunteered on before. It was good to spend time talking and getting to know each of the young people and I was inspired by their positive attitude towards life, especially with the many challenges that they face and overcome every day.

I've really enjoyed volunteering at the MPS weekends, getting to meet some amazing people, experience some really interesting situations and have a great time! MPS weekends are definitely the most exhausting weekends of my year but also some of the most rewarding and fun ones too. **Hannah Donegani**

Volunteering opportunities at MPS

Are you interested in becoming a childcare volunteer for the MPS Society? Perhaps you're not able to support us through fundraising, but maybe you can spare some time and energy to be one of our childcare volunteers? Maybe your work place and colleagues could donate some time to support our childcare programme at events?

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

To become a childcare volunteer we would need you to complete an application form. 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. Once we have obtained satisfactory references and your CRB check, you will then receive an acknowledgement that your application has been accepted and you will be added to the volunteer mailing list. All new volunteers will be mentored by an experienced volunteer.

The MPS Society organises a programme of events and activities throughout the year. These include sibling weekends for brothers and sisters of children affected by MPS and related diseases, adult weekends for adult individuals affected by the diseases and family weekends, expert meetings and conferences for the whole family to participate in.

Our event programme is exciting and we rely on our childcare volunteers to keep our children and vulnerable adults safe whilst ensuring they have a happy and memorable time.

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

Spring 2012

Society for Mucopolysaccharide Diseases

mps children's newsletter

Welcome! Thank you so much to everyone who sent in the stories and pictures that appeared in the last issue. It was great to hear from all of you!

As always, we really want to hear from children that are affected by MPS as well as their brothers and sisters. You can get in touch and have your stories, poems, drawings or messages in the next edition of the MPS Children's Newsletter.
You can email us at magazine@mpssociety.co.uk but don't forget to ask permission first!

Check out our Children's Section on the MPS website www.mpssociety.co.uk
Don't forget to ask permission first!

what's inside...

Can you help MPS raise money whilst getting fit & having some fun?

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 but here are a selection:

BUPA Junior and Mini Great Manchester Run
Saturday 19 May 2012
Distance: 1.5 - 2.5km
Age categories: 3-8, 9-14 years

BUPA Junior and Mini Great North Run
Saturday 15 September 2012
Distance: 1.5 - 4km
Age categories: 3-8, 9-10, 11-12, 13-14, 15-16 years

BUPA Junior and Mini Great Edinburgh Run
Sunday 7 October 2012
Distance: 1.5 - 2.5km
Age categories: 3-8, 9-14 years

BUPA Junior and Mini Great South Run
Saturday 27 October 2012
Distance: 1.5 - 2.5km
Age categories: 3-8, 9-15 years

If you get a place on one of these runs, please let us know. We will send you a fundraising pack to help you get your friends, family and school involved!
E: fundraising@mpssociety.co.uk

Ask your parents or guardians to contact us for more information!

Naomi's Story

Hi, my name is Naomi. I am 12 years old and I have Fabry disease...

Naomi having her enzyme replacement therapy

Having Fabry takes up a lot of time. I have to go to lots of hospital appointments. Some of them are at Great Ormond Street Hospital and some are nearer to my home in Cornwall.

I have an enzyme replacement (ERT) infusion (drip) every two weeks. This used to be in hospital but now we have a lovely nurse, Cherrie, who comes to our house. My mum has been taught how to put the cannula in.

I have medicines for my Fabry disease. Fabry makes my hands hurt and I have pain in my joints, head, neck and back. For pain I take paracetamol and other medicines. Sometimes the Fabry makes me have tummy problems where I get painful cramps and I need the toilet alot.

Being on the ERT has improved my symptoms so much. I hardly ever need to take immodium and my pains are better. Fabry disease also makes me dizzy - I think it is called vertigo but it isn't about being scared of heights. For the dizziness I take medicine too.

After my infusion I have more energy and feel lots better, but by the time I need my infusion I'm in lots of pain and am very tired. It would be nice if the infusion would make me completely better but since I started on it last May I feel much happier!
Naomi Carter

Blaise and his award

Congratulations to Blaise for winning award!

Congratulations to Blaise Leslie on his award from the Princess of Wales Trust. The award was for a short film Blaise helped to make with a local high school called 'diversity'

MPS SIBLING FACT SHEET
AvAILABLE NOW!
Visit www.mpssociety.co.uk
for more information

Getting involved with MPS

There are loads of ways that you and your family can get involved with the MPS Society. Perhaps you could do some fundraising with your family, friends or at school. Read over the page to see how you can help us on MPS Awareness Day. Or write something for our MPS magazine or children's newsletter. Or why not join us at our events?

Having a meal together

Manchester Family Day

Here are a couple of pictures from the Manchester Morquio trial family day in January. They had a great day and said a big thank you to MPS for organising it.

Outside the grounds

MPS Sibling Week

Children with MPS brothers and sisters came together in July 2012 for a week filled with fun activities. Everyone made some good friends and had a fantastic laugh!

MPS Siblings

Ask your parents or guardians to look out for information about our MPS events in the MPS magazine and on our website www.mpssociety.co.uk. We would love you to join us for some fun!

Don't forget that you can send in your photos, drawings, jokes, stories, poems, questions or suggestions for what you'd like to see included in these pages. Ask your parents or guardians to send these to us by post, or by email at magazine@mpssociety.co.uk

We have a number of **children's booklets** to help you learn about and understand MPS diseases and help explain them to others.

MPS Awareness Day

Can you help us?

The MPS Society always needs lots of help from people all around the country to raise money to help children and grown ups that are affected by MPS or related diseases.

Every year we have an MPS Awareness Day where we encourage everybody to spread the word about who we are and what we do!

The next MPS Awareness Day will be on Tuesday 15th May 2012.

We are asking everybody to use this day to tell everyone they know about MPS, whether you're at school or with your family and friends. MPS Awareness Day is all about getting the word out to anyone and everyone.

Why not be really brave and ask if you can tell all of your school friends and teachers about the MPS Society in a school assembly?

Visit our website www.mpssociety.co.uk to download one of our school presentations. Click on the 'Downloads' link on the left hand side of our homepage and then click on 'Fund-raising Resources'. Here you can choose from three presentations.

Another way you can help raise awareness of the MPS Society is by organising a fundraising event or activity.

By fundraising you will be able to raise money and awareness all at the same time.

You could try a walk, a run or a cycle or you could have a talent competition, a bake sale or a sports day in aid of the MPS Society.

You could also write to your local newspaper to tell them all about what you are doing, or you could write to them afterwards and send in some pictures.

We have an MPS School's Fundraising Pack that we can send to your parents or guardians or your school. It's full of MPS posters, stickers and balloons and has all the information that your school will need to know.

If you are planning on doing something to raise awareness for the MPS Society, we would love to hear all about it. Your story could appear in our special Fundraising Magazine or in the next issue of the MPS Children's Newsletter!

Society for Mucopolysaccharide Diseases MPS House, Repton Place, White Lion Road, Amersham, Buckinghamshire, HP7 9LP
T: 0845 389 9901, F: 0845 389 9902, E: mps@mpssociety.co.uk, **www.mpssociety.co.uk**
Registered Charity No. 1143472 Charity Registered in Scotland SCO41012

Fabry Focus Groups

The focus groups were organised by the MPS Society, and hosted by Professor Mehta at the Royal Free and Professor Cox at the Sidney Sussex College, Cambridge. The aim was to update the patients on treatment options and to explore the challenges and expectations of individuals with Fabry Disease. Patient representation came from Addenbrookes, Royal Free and The National Hospitals.

Fabry Focus Groups held at the Royal Free Hospital, London & Sidney Sussex College, Cambridge on 20th & 21st May 2011

Our apologies for the delay not presenting the information until now but this was due to unexpected work pressure from the transcribing company.

The days started with presentations from the Professionals and after lunch the groups were asked a number of questions and asked for their observations. The following narrative data and conclusions were drawn up after analysing the information following the focus groups.

Royal Free Hospital, London
The questions and conclusions are listed below:

Q1 What are the challenges of living with Fabry, from a school, college, work, social point of view?

Conclusion
There were a number of observations regarding how tiring it is for patients to have to attend different hospitals for different tests and a desire that all appointments be made in one day. Some of the patients did not attend The Royal Free and this comment is likely to have been from patients attending other hospitals.

The challenge of sustaining relationships due to dealing with the impact of Fabry disease and at the same time trying to continue a 'normal' life was very prevalent. Trying to balance family life and coping with the disease was acknowledged as challenging. The support provided by a partner depended on the relationship of each couple.

Negative impacts were identified between mother and child if the child has to be a 'carer'.

There is considerable impact on the family when a diagnosis had not been established. An older family had great difficulty when their children were at school due to the disease not being diagnosed. The children had a lot of time off school and this raised child protection issues and resulted with psychiatrists and psychologist's becoming involved until there was an eventual Fabry diagnosis.
This can have a devastating effect on the family unit before diagnosis. Parents clearly know something is wrong but may not be taken seriously?

The impact on young people as to how they are perceived by others has a profound effect on them.
It raises the question as to whether more could be done to help younger patients deal with the emotional aspects of being a teenager and having Fabry Disease?

Not being able to keep up with peers and been branded 'lazy' is identified as having a detrimental impact on the young Fabry patients.

Q2 Are my expectations of Clinical Management being met?
What is your experience of support from the medical teams, feedback on test results etc.?

Conclusion
Generally patients are happy with the LSD service they receive.
Some patients reported that not all AGNSS centres may have been as approachable in the past when patients have phoned for advice, but that situation is changing.

Patients like to meet the same group of patients when they attend for their appointments as this helps to build up a rapport. Mutual support from other people who have Fabry is very important.

Q3: Are my expectations of the benefits of ERT being met?
Where did you find out about the effects of the therapy, were you told about possible side effects etc.?

Conclusion
Fabry information and literature provided, focus groups, conferences, MPS Magazine and the MPS Society are all received favourably by patients.

Generally each person deals with the diagnosis, and the information processing in their own way.
Some patients may want to know everything whilst others only want to know the basics. Providing on-going information as the patients feel they need to learn more is essential

Receiving ERT on the whole, had a good psychological impact on patients even if symptoms are not stabilised. Patients identified that having the treatment was better than not having any treatment.

Q4 How can services improve from a clinical patient support perspective? Any problems with home infusions, going to clinics for routine tests, etc.

Conclusion
The majority of patients liked the fact that the tests are carried out on one day.

It was felt that meeting the doctors in a more informal way i.e. conferences, information days, was a positive experience that led to even more positive clinical experiences.

On the whole the nurses do get back to patients if they have a query but not always as quickly as patients would like.

Further consideration may need to be given to the waiting times patients endure between tests and when patients are to see the Consultant.

How Services might be Improved
Could patients be advised to come back at a certain time so they are able to go and get either something to eat or go for a walk?

Patients would like to have the test results letter sent to themselves as well as the GP.

Patients would like to be able to discuss their test results with the Consultant, rather than the GP. They would like to have a note put in the letter about whether they should worry about any changes in the test results.

Patients find it difficult having to explain Fabry disease to non-specialist doctors, however there is information on the MPS website and the Patient Information Cards are available to members of the MPS Society. These contain information such as the patient number, disease, the centre were they are registered, the Consultants details and contact number. Specialist Centres could promote this.

Sidney Sussex College, Cambridge
The questions and conclusions are listed below:

Q1 What are the challenges of living with Fabry from a social, school, college, work point of view?

"I don't want to be treated differently really, so I don't really tell anyone that doesn't have to know that I have Fabry"

"At school it only affected me a bit, with sports, but I tried not to let it"

"Socially I don't usually go out if I am not well"

"I was diagnosed when I was small so I have grown up with the disease, I don't know anything different"

"My ERT is just part of my routine, and part of my life"

Conclusion
All of the general comments came from a young person and although Fabry has been part of her life for many years she still only lets a select number of friends know of her condition. There is a need to consider the impact of Fabry Disease on young people and what support may be available to help them.

The negative effects on a non-sufferer in the family are difficult to quantify and yet these people are integral to the wellbeing of the sufferer especially as they see their family members having to deal with the effects of the disease.

Q2 Are my expectations of Clinical Management being met:
What is your experience of support from medical teams, feedback on tests results etc.?

Conclusion
Generally patients valued the opportunity to attend and participate in Conferences and Focus Groups as these events helped to keep them up to date with clinical management, treatment options and outcomes as well as research initiatives.

Patients clearly identified the need for test results to be more clearly presented allowing the patient to make comparisons with previous test results. Patients need to understand the clinical implications of any changes in test results. Whilst it is common practice, some patients raised concern that they do not receive a copy of the letter sent to their GP following hospital visits.

Patients identified the need for feedback from the Consultant and to answer their questions in an open and transparent manner.

Patients felt that the GP's were reluctant to treat them, even if the problem was not related to the Fabry Disease; possibly more communication is needed between the GP's and the Consultants.

Having someone available at the specialist centre to answer any questions or queries from a patient is important, whether this is by phone or e-mail.

Q3: Are my expectations of the benefits of ERT being met?
Where did you find out about the effects of the therapy, were you told about possible side effects etc.?

Conclusion
Generally speaking most of the patients went on treatment because they hoped it would make them feel better and slow down any progression of the disease.

It was accepted that there is more information now about the disease and benefits of treatment now particularly when women were previously only acknowledged as carriers and not symptomatic for the disease. This was very apparent to patients who were diagnosed over 15 years ago. Patients agreed that in general they are now more empowered to speak freely to their clinicians and ask questions.

A majority of patients would welcome a simpler less invasive method of treatment than Enzyme Replacement Therapy (ERT) but recognise that Chaperone treatment will only be able to help a small percentage of patients.

ERT appears to have caused some relationship problems when the partner would not accept or want to acknowledge that their partner was ill.

Patients felt that more support was needed when there was a new diagnosis and this also goes hand in hand in looking after the patient's on-going mental health and wellbeing as time goes on and the disease progresses.

Some asymptomatic patients expressed guilt for the costs of their treatment. A number of participants voiced the cost of treatment is a consideration for them from a societal point of view.

Q4 How can services improve from a clinical patient support perspective? Any problems with home infusions, going to clinics for routine tests, etc.

Conclusion
Patients appreciated that they could get their treatment at home and they agreed that the nurses were all really good.

The deliveries from the Homecare Services of the drugs and equipment seemed to be a problem for patients. The problem areas appeared to be over deliveries not being on time. This problem escalated when one of the Homecare Companies changed hands creating a lot of problems. Patients prefer to have the same delivery driver if they can, apart from sick absences and holidays. The driver tends to get to know the patient and the family and this makes things easier and results in a satisfactory delivery. There is a need for the delivery company to accommodate any changes to plans.

A concern was raised over the delivery company not keeping to their contract with the hospital and advising the hospital of various targets to be met which from the patient's perspectives are not being achieved.

Patients attending the Focus Group advised that they had not received any form of questionnaire in relation to the Homecare Service. Patients and the MPS Society agree that a satisfaction survey should be carried out at least once a year by an independent organisation. It is clear patients are concerned that the Hospital and Homecare Managers may not be getting a true picture of the service they are paying for or managing.

Patients appreciate having Conferences and Information Days to keep them updated and enable them to meet other Fabry sufferers and their families.

Some patients felt that the partners were sometimes forgotten and they also need support. Patients were advised of the MPS Society's Befriending Scheme, which offers to put members in touch with other member's for mutual support.

Thank you everyone for making the events a success.

Rebecca Brandon
r.brandon@mpssociety.co.uk

Emergency Patient Information Cards Update

We are continuing to receive application forms for the Emergency Patient Information Cards and the feedback is still very positive.

There is one letter in particular that we have received that we would like to share with you. This letter carries the exact message that we would like to get across to our members about how important the cards could be:

"We have had the form for the Patient Information Cards for a while and thought that would be a good idea but as we only attend Great Ormond Street Hospital we thought we didn't want to bother anyone.

This was until we had to take Harry to Stoke Mandeville Hospital before Christmas. When he was discharged on his notes it said he suffers from Hunters Syndrome [Harry has Hurlers]. You know as we do that the syndromes are different and that treatments or drugs could possibly harm the children involved.

So we thought that it would be a good idea to ask you if it would be possible to make us one of the cards, so that there can be no doubt in the future if we have to attend any hospital other than Great Ormond Street Hospital."

This email was received by the same family after they had received the card:

"After what happened when we had to take Harry to hospital, I would urge all parents to carry these cards to ensure that their children get the right medical treatment and any possible problems that could arise eliminated by the simple reading of a card. We really could not believe that something so important could be so wrong."

This is, of course, applicable to all our members, adults as well as children, should they need to attend a different hospital from the one they are registered with.

An example of how the card looks is shown here. The image shows both the front and back of the card. Each card is unique to the individual and the particular MPS disease they have.


This patient suffers from a Mucopolysaccharide or Related Disease

Name: A Sample
Date of Birth: 00/00/0000
MPS Disease: MPSIV Morquio
Hospital: Royal Manchester Children's Hospital Oxford Road, Manchester, M13 9WL
Hospital Tel No: 0123 456 7890
Hospital Number: 123456789
Consultant: Dr Sample

Society for Mucopolysaccharide Diseases

Important Medical Information

MPSIVA (Morquio) is a rare genetic disease associated with significant short stature, physical disability and, in many cases, limited life expectancy. Significant skeletal problems include instability of the cervical spine which may have been treated by a cervical fusion. There is currently no therapeutic treatment, however some patients may be on a clinical trial for Enzyme Replacement Therapy.

Urgent Patient Specific Information:

Cervical instability
Intravenous Enzyme Replacement Therapy

MPS Society Number: 08453 000901
www.mpssociety.co.uk

MPS Society Out of Hours Office Number: 07712 653259

If you are interested in having a card for you and/or your child(ren), please download an application form from our website. If you do not have access to the internet, you can request an application form by phone on 0845 389 9901.

Additional cards are available but may incur a small charge; please contact us for more information.

Transitioning from paediatric to adult LSD clinical services: what are our choices?

We are often being asked by our members about choices around transition from paediatric to adult LSD clinical services. This question comes from teenagers and parents of teenagers facing a move to an adult LSD clinical service. The MPS Society's position is quite clear and is the line taken by the Senior Commissioners for National Commissioned Services (NCG) in England. Whilst it may be anticipated that children transitioning from paediatric services in Manchester and Birmingham will go automatically to the LSD adult service in the same city they don't have to. In London children transitioning from the LSD service at Great Ormond Street already have a choice of the National Hospital, Queen's Square or the Royal Free Hospital, Belsize Park. Whilst Addenbrookes Hospital, Cambridge does not have an NCG LSD paediatric service children can transition from any of the LSD paediatric centres to Addenbrookes Hospital for NCG LSD adult services.

SO WHAT ARE MY CHOICES OF NCG LSD CLINICAL CENTRES?

Salford Royal Foundation Hospital
Dr Reena Sharma; Dr Ana Jovanovic ; Dr Chris Hendriksz (from 1/3/2012)
Address: Salford Royal Hospital NHS Foundation Trust, Department of Adult inherited Metabolic Diseases, Stott Lane, Salford, Greater Manchester, M6 8HD
Tel Contact :- 0161 2064365

Queen Elizabeth Hospital
Dr Tarek Hiwot
Address:- University Hospital Birmingham NHS Foundation Trust, Queen Elizabeth Hospital, Queen Elizabeth Medical Centre, Birmingham, B15 2TH
Tel contact:- 01223 274 634

Addenbrookes Hospital
Prof Tim Cox; Dr Patrick Deegan
Address:- Cambridge University Hospital NHS Foundation, Department of Medicine, Hills Road, Cambridge, CB2 0QQ
Tel contact:- 01223 245151

National Hospital
Dr Robin Lachmann; Dr Elaine Murphy
Address:- National Hospital for Neurology & Neurosurgery, Charles Dent, Metabolic Unit, Queens Square, London, WC1N 3BG
Tel contact:- 0207 829 8778

Royal Free Hospital
Prof Atul Mehta; Dr Derralynn Hughes
Address:- Royal Free Hampstead NHS Trust, Pond Street, London, NW3 2QG
Tel contact:- 0207 7940500

HOW DO I CHOOSE?

- The first place to start is by discussing your thoughts with your / your child's paediatrician
- Consider arranging to meet the adult team(s) outside of a clinical appointments of one or more centres you might be interested in
- Have a list of questions that may be around clinical management, treatment, in patient and out patient facilities and family logistics
- You should never feel under pressure to go to a specific centre and all centres accept that from time to time for a variety of reasons a patient may move to another centre
- If you have any concerns about transition you can talk these through with a member of the advocacy team. However the advocacy team will not advise you of which centre to go to.

THE MPS SOCIETY AND TRANSITION

As many of you will know the MPS Society with the other LSD Patient Associations are funded with a QIDIS grant to develop a 'Model of Transition for LSD Patients'. We are in the final stages of this project being carried out by Lindsey Wingate and managed by Sophie Thomas. We are expected to report to the National Commissioning Group imminently and expect to be able to report the outcomes to our members later in the year.

Eye care for children at Great Ormond Street Hospital

In November 2011 Mr Ken Nischal, Consultant Ophthalmic Surgeon at Great Ormond Street Hospital left to take up a new post in the United States and the department is now led by Dr Alki Liasis, a first class visual scientist who plans to develop the department in the same manner. Mr Will Moore has been especially appointed to look after children with cornea, cataract, craniofacial and glaucoma problems. A further surgical appointment is planned.

Mr Nischal has helped many MPS patients at Great Ormond Street Hospital as well as share his expertise at several of our National MPS Conferences. We are sorry to see him depart but wish him every success in his new position

Christine Lavery
c.lavery@mpssociety.co.uk

Formal Apology

"The National Specialised Commissioning team would like to apologise formally to the Cambridge University Hospitals (CUH) NHS Trust for some statements contained in the article in the last issue of the newsletter about the paediatric lysosomal storage disorder service. The National Specialised Commissioning team wish to record formally that there were no concerns regarding patient safety or the quality of the service provided at CUH. The decision to decommission the service at CUH was taken in full agreement with the executive at CUH on the grounds of low numbers of patients with LSD who were being managed by the service. This correctly led to the agreed conclusion that the paediatric LSD service at CUH was not sustainable and it was therefore decommissioned. We regret any statements in the article which misrepresented the facts and apologise to all of the dedicated and highly professional colleagues who provided the service at Cambridge prior to its decommissioning for any offence or distress caused."

National review of purchasing arrangements of high cost drugs and homecare services for lysosomal diseases

The National Specialised Commissioning Team (NSCT) has commissioned work to review the procurement and delivery of Enzyme / Substrate Replacement Therapies in the Lysosomal Storage Disorders service.

The project is being undertaken in collaboration with the Commercial Medicines Unit (CMU) at the Department of Health and East & South East England Specialist Pharmacy Services team and comprises of two elements.

- National tender for the treatments of lysosomal storage diseases
- National tender for the delivery of home care for patients on enzyme / substrate replacement therapy

It is unsurprising that in the current economic climate that a National Framework is being developed rather than a centre by centre tendering approach. Currently the LSD service budget is £8 million and the combined drug and home care budget is £128 million.

The key objects of this framework will be to improve governance and achieve better value for money so that as many patients as possible in the future will be able to benefit. Along with LSD specialist clinical centres in England the MPS Society and other LSD patient associations are being consulted. It is anticipated that this new framework willcome into operation on 1 May 2012.

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Expansion of ongoing study 'An investigation into sleep, circadian rhythm, behaviour and family'

Thank you to all the families who have taken part in this ongoing study. We have been really excited by the responses we have received so far and hope to be able to publish our results by September 2012.

We are now expanding our study to include families who have a son or daughter with MPS III Sanfilippo who is older than 15 years of age. So, we would like to invite all families with a son or daughter who is two years of age or older to participate.

This will involve spending between 1 to 1 and a half hours completing questionnaires relating to behaviour, development and family functioning. Questionnaires will be sent and returned by pre-paid registered post. You will have the option of completing the questionnaires independently or with a researcher.

If you would like to find out more about taking part in our study please contact us by email elaine.cross@postgrad.manchester.ac.uk or phone 079 83759667.

Elaine Cross, Sheena Grant, Louise Mahon and Michelle Lomax
Trainee Clinical Psychologists
University of Manchester

Amicus Therapeutics announces Positive Preliminary Results from Ongoing Phase 2 Chaperone-Enzyme Replacement Therapy (ERT) Study for Fabry Disease

Co-Administration Increases Levels of Active Enzyme Compared to ERT Alone in First Six Patients

CRANBURY, NJ, USA, January 6, 2012 – Amicus Therapeutics (Nasdaq: FOLD), today announced preliminary results from an ongoing, open-label Phase 2 drug-drug interaction study (Study 013) to evaluate the safety and pharmacokinetic (PK) effects of two doses of migalastat HCl (150 mg and 450 mg) co-administered with ERT (agalsidase beta or agalsidase alfa) in up to 24 males diagnosed with Fabry.

Amicus and GSK are developing migalastat HCl, an investigational oral pharmacological chaperone, as part of a global collaboration for Fabry disease. Migalastat HCl is in Phase 3 development (Study 011 and Study 012) for use as a monotherapy for patients with Fabry disease identified as having alpha-galactosidase A mutations amenable to chaperone therapy.

When co-administered with ERT, migalastat HCl is designed to bind to and stabilize the enzyme in the circulation, in any patient receiving ERT. In preclinical studies, the co-administration of migalastat HCl and ERT led to stabilization of the ERT and increased uptake of active enzyme into key organs of disease, including kidney, heart, and skin, when compared to ERT alone. This increased enzyme uptake in Fabry mouse models also led to further reductions in globotriaosylceramide (GL-3), the substrate that accumulates in kidney, heart and skin in Fabry disease.

Data are currently available for the first six subjects in Study 013, who received their current dose and regimen of agalsidase beta alone at one infusion followed by oral migalastat HCl 150 mg administered two hours prior to agalsidase beta at their next infusion. Due to the supply shortage of agalsidase beta, four of these subjects had been receiving 0.5 mg/kg infused every two weeks and two subjects had been receiving a dose of 1.0 mg/kg infused every four weeks.

Preliminary Results - Migalastat HCl 150 mg Co-Administered with Agalsidase Beta (n=6)

- Increases in levels of active enzyme in plasma and skin demonstrate a positive drug-drug interaction between migalastat HCl 150 mg and agalsidase beta
- In the four patients who received agalsidase beta at 0.5 mg/kg co-administered with migalastat HCl 150 mg, levels of active enzyme in plasma ranged from 2.0 to 4.2-fold higher than with ERT alone, as measured by total area under the curve (AUC). In skin biopsies from three patients, increases in levels of active enzyme in the skin ranged from 1.1 to 3.9-fold higher at day two, but no higher at day seven, following co-administration compared to ERT alone.
- In the two patients who received agalsidase beta at 1.0 mg/kg co-administered with migalastat HCl 150 mg, levels of active enzyme in plasma were 1.6 and 2.2-fold higher than with ERT alone, as measured by total AUC. In skin biopsies, increases in levels of active enzyme in the skin were 1.6 and 2.1-fold higher at day two, and 1.2 and 1.7-fold higher at day seven, following co-administration compared to ERT alone.
- Preliminary data to be presented as a "late breaking" abstract at 8th Annual Lysosomal Disease Network WORLD Symposium (LDN WORLD) in San Diego, February 8-10, 2012.
- Independent data safety monitoring board approved dose escalation to migalastat HCl 450 mg as per study protocol. Amicus and GSK expect to complete Study 013 in the first half of 2012.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics said, "These very encouraging data in Fabry patients represent an important first step in validating the potential of pharmacological chaperones to enhance the stability and tissue uptake of enzyme replacement therapy products in Fabry disease. We look forward to completing Study 013, including obtaining data on the higher dose of migalastat HCl as well as data on coadministration with agalsidase alfa. We also look forward to evaluating the complete data set and collaborating with GSK on the best path forward for extending this co-administration use of our core technology as a treatment option for people living with Fabry disease."

Amicus Therapeutics Presents Patient Screening Profiles From Phase 3 Fabry Study

Majority of Subjects Screened as of July 2011 had Missense Mutations Considered Amenable to Migalastat HCl Monotherapy Based on an in Vitro Assay

CRANBURY, N.J., Feb. 13, 2012 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, recently presented updated information on the number and type of patients screened for and enrolled in the global Phase 3 registration study (Study 011) of the investigational pharmacological chaperone migalastat HCl, being studied as a potential monotherapy for Fabry disease.

At the 8th Annual Lysosomal Disease Network WORLD Symposium (LDN WORLD), Dr. Daniel Bichet, Universite de Montreal, gave an oral presentation titled, "Fabry Disease Mutations Addressable With Migalastat HCl, an Investigational Chaperone Therapy. Screening Results from FACETS, a Phase 3 Study in Male and Female Patients." Key findings presented by Dr. Bichet include:

- 140 Fabry patients (46 males and 94 females) with mutations in Alpha-galactosidase A (alpha-Gal A) had been screened for Study 011 as of July 2011
- Approximately 85% (119/140) of the patients screened carried missense mutations
- Approximately 82% (97/119) of those patients met one of the key enrollment criteria of having amenable mutations and were potentially eligible for the study.

Dr. Bichet stated, "A majority of the first 140 subjects screened in Study 011 had missense mutations, and most of these mutations were also amenable to migalastat HCl as a monotherapy. Given the entry criteria for Study 011, we generally screened patients that we believed were likely to have missense mutations. The clinical significance of having these mutations is unknown and can only be established in studies such as Study 011. We continued to gather data among patients screened through the close of enrollment, and look forward to sharing additional findings at a future scientific congress."

Update on Patients Enrolled in Study 011

Amicus and its collaborator GlaxoSmithKline (GSK) have enrolled 67 (24 males and 43 females) out of 180 total subjects screened in Study 011. The study completed enrollment in December 2011. All patients enrolled in the study have been diagnosed with Fabry disease, and were naïve to enzyme replacement therapy (ERT) or had not received ERT for at least six months prior to study entry.

In addition, each patient enrolled in Study 011 met further entry criteria, which included having alpha-Gal A mutations amenable to migalastat HCl monotherapy in vitro. For study purposes, all patients also needed to have urine globotriaosylceramide (GL-3) at least four times the upper limit of normal. A number of patients screened had amenable mutations but did not have sufficiently elevated urine GL-3 at baseline to qualify for enrollment into Study 011.

Elevated levels of GL-3 in urine have been shown to correlate with GL-3 levels in kidney tissue, including the interstitial capillaries. The primary efficacy endpoint for Study 011 is a change in interstitial capillary globotriaosylceramide (GL-3) as measured in kidney biopsies. Patients in Study 011 with a reduction of GL-3 deposits per capillary of at least 50% at six months will be considered responders.

John F. Crowley, Chairman and Chief Executive Officer of Amicus stated, "We are pleased that the 67 Fabry patients enrolled in Study 011 include a range of patients by gender, disease burden and genotype who are living with Fabry disease today. Study 011 is the first randomized, controlled Fabry study to ever include a large number of female patients, who represent more than half of all patients in Fabry disease registries. We look forward to seeing these study results in the third quarter of this year."

Migalastat HCl (AT1001, GS181413A) physician initiated request program for Fabry disease

We write to update you, as you have requested, with news about a migalastat HCl (AT1001, GS181413A) physician initiated request program for Fabry disease that recently became available in December.

Program MGM116188: Physician Initiated Request for Treatment Use of Migalastat Hydrochloride (GR181413A/AT1001), an Investigational Treatment for Individual Patients with Fabry.

This Physical Initiated Request program allows physicians to request GSK permission to receive migalastat HCl for eligible patients with Fabry disease who do not meet requirements for participation in an existing migalastat clinical study. Up to 20 patients worldwide may be treated. Patients must meet specific criteria to receive GSK permission for participation. Among these criteria are an underlying Fabry disease mutation that may be responsive to migalastat HCl and the lack of any other available treatment option (because the patient is either unsuitable for or unable to access enzyme replacement therapy [ERT]).

Requesting physicians must have served as an investigator in a migalastat HCl study. The requesting physician has to obtain Institutional Review Board (IRB)/Ethics Committee (EC) approval and regulatory approval prior to initiation of treatment. Key criteria for participation are detailed on www.gsk-clinicalstudyregister.com or http://clinicaltrials.gov.uk/show/NCT01476163

Please do not hesitate to contact your Fabry physician if you have any questions about this physician initiated request program. Thank you for your continued interest.

Genzyme Announces FDA Approval of Framingham Manufacturing Plant

CAMBRIDGE, Mass. – Genzyme, a Sanofi company (EURONEXT: SAN and NYSE: SNY), announced today that the Food and Drug Administration (FDA) has approved its manufacturing plant in Framingham, Mass., for the production of Fabrazyme® (agalsidase beta). This follows the previously announced approval by the European Medicines Agency (EMA) last week.

"We are very pleased with the FDA approval of our Framingham plant as we continue our manufacturing recovery and path forward to serve the Fabry patient community," said Genzyme's President and CEO David Meeker. "With this approval, we continue upon our 2012 plan to restore unconstrained supply for all patients globally throughout the course of the year."

Approval of the Framingham site allows Genzyme to begin the process of returning patients to full dosing (1 mg/kg) levels. Following the EMA approval, Genzyme will begin the process of moving the most severely affected patients in Europe to full dose of Fabrazyme in Q1 2012.

Beginning in March, all patients in the U.S. currently on therapy will be returned to full dosing. In addition, the company will begin to transition new patients in the U.S. onto Fabrazyme, at full dosing levels. Globally, the complete return to normal supply levels of Fabrazyme will begin in the second quarter and continue throughout the year as planned, as Genzyme works to obtain all global regulatory approvals throughout the year and to build inventory.

Shire Human Genetic Therapies (HGT's) public announcement regarding our program for Sanfilippo B syndrome (MPS IIIB)

We are excited to inform you about Shire Human Genetic Therapies (HGT's) public announcement regarding our program for Sanfilippo B syndrome (MPS IIIB). When know that there will have been many enquiries from families regarding Shire HGT and Sanfilippo syndrome; therefore, here is an update for members.

As you may have learned, Shire HGT is planning to sponsor a natural history surrogate endpoint trial for individuals with MPS IIIB at a small number of sites around the world. When the study is initiated, the contact information for the selected sites and details of the inclusion and exclusion criteria will be posted on www.clinicaltrials.gov. We expect this study will be initiated in February 2012 and sites will be actively recruiting for interested patients. As there is currently no approved treatment for MPS IIIB, this natural history study will serve to provide a more robust understanding of the disease and guide efforts toward the development of a therapeutic intervention.

With the announcement of Shire HGT's MPS IIIB programme, we continued our commitment to rare genetic diseases, and we are grateful for the opportunity to work with the MPS community in our mission to be as brave as the people we help.

Fabrazyme Shortage

After a very difficult time for patients with Fabry disease globally it is very good news that Genzyme's new manufacturing plant at Framlingham, Boston, USA, has received FDA approval and the Fabrazyme inventory of the drug is being built up. The first to benefit will be Fabry patients in the USA who have not had the benefit of being able to switch to Replagal. Once the initial demand of US Fabry patients to receive Fabrazyme is being met, the focus will turn to Europe and the rest of the world. Equally Shire is awaiting approval of Replagal by the FDA for use in the USA.

Fabry patients might to wish to be aware that there is an intiative by some European LSD/Fabry expert clinicials to draw up a paper with recommendations on prioritising the distribution of agalsidase beta (Fabrazyme) in the European Union. The Fabry International Network Board (FIN) have been given the opportunity to read and reflect on the paper and have offered some feedback. FIN is also aware of substantive comments from clinicians. Unfortunately, at the time of going to print I have not seen a final document and do not know whether the points made by FIN or other clinicians are reflected in the final draft recommendations on prioritising increase of agalsidase bea in the EU to published shortly.

On reflection, the MPS Society would offer a few thoughts as bullet points to help our Fabry members who have been affected by the shortage of Fabrazyme.

- You do not have to switch from Replagal to Fabrazyme however your clinician may suggest the switch for clinical reasons.
- If your clinician does propose a switch from Replagal to Fabrazyme do reassure yoursef of the clinical rationale behind the proposal by asking your clinician.
- If you are on Replagal and you would like to switch to Fabrazyme do discuss this with your clinician.

Broadly speaking pan European recommendations might by useful in some countries. However, we are most fortunate that the UK has a very joined up Fabry service with a number of paediatric and adult clinical leads in the field who will be best placed to work in partnership with you to ensure the Fabry treatment regime you receive is appropriate for you.

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Shire Announces European Approval of Manufacturing Facility for VPRIV® (velaglucerase alfa)

European Medicines Agency Approval Adds Significant Capacity for the Manufacture of Shire's Enzyme Replacement Therapies

Dublin, Ireland – February 22, 2012 – Shire plc (LSE: SHP, NASDAQ: SHPGY), the global specialty biopharmaceutical company, announced today that the European Medicines Agency (EMA) has approved the production of VPRIV® (velaglucerase alfa) in its new state-of-the-art manufacturing facility at 400 Shire Way in Lexington, MA. The European Commission's decision is expected imminently.

"We welcome the news that Shire's new manufacturing facility in Lexington has received EMA approval for the production of VPRIV," said Tanya Collin-Histed of the UK Gaucher Association. "This provides patients with greater comfort over the maintenance of supply of enzyme therapies for the treatment of Gaucher disease."

"The EMA approval of the VPRIV manufacturing facility will give confidence and reassurance to patients with Gaucher disease," said Professor Timothy Cox, Department of Medicine, University of Cambridge. " Increasing the availability of enzyme replacement therapy will be of great comfort to patients in the UK and across the world."

Shire now has two EMA approved facilities – Alewife, in Cambridge, MA, as well as the new Lexington facility – in which to manufacture VPRIV drug substance. This additional capacity will allow Shire to significantly increase global supply of VPRIV and provides additional manufacturing flexibility. The EMA approval is also a critical first step in releasing further capacity for the manufacturing of REPLAGAL® (agalsidase alfa) at Shire's Alewife facility. The new facility increases bioreactor capacity from 1000 to 8000L, and is the first commercially licensed facility in the world to utilize single-use bioreactor and disposable technology throughout cell culture processing to reduce manufacturing risk.

"I am delighted to announce the EMA approval of our facility. Shire has invested strategically in new manufacturing facilities and state-of-the-art technology because we recognize the critical importance of ensuring the continuity of treatment for patients with rare and life-threatening diseases," said Bill Ciambrone, Senior Vice President, Technical Operations, Shire HGT. "The EMA approval of VPRIV in this manufacturing plant, only three years after breaking ground, is a testament to the hard work and dedication of Shire employees, and represents crucial additional capacity for manufacturing our enzyme replacement therapies for Gaucher and Fabry patients."

WORLD Symposium
8 - 10 February 2012, San Diego, California

The WORLD Symposium is the annual research meeting of the Lysosomal Disease Network. It is arguably the most important multidisciplinary forum in the LSD community calendar presenting the latest information from basic science to translational research and clinical ntrials for MPS and other related lysosomal storage diseases. The first WORLD Conference was conceived in 2004 and such is the popularity of this meeting that registrations have grown from 350 in in 2009 to nearly 600 for the 2012 meeting. The theme 'transitioning from translation to trials' seeks to elucidate the challenges and successes in bringing bench discoveries into successful clinical therapies. This year the programme was assmbled almost entirely from submitted research abstracts with only three invited speakers. This allowed for a larger and more diverse representation of the work being carried out in the field. The first day was focused on basic research, the second day on translational research and the third on clinical research. This day by day progression through the three stages of research is a feature of the WORLD conference and allows those attending to plan their networking meetings around the relevant presentations and poster sessions.

Presentations in Basic Research included:

- Excessive Activity of Cathepsin K is Associated with the Cartilage Defects in a Zebrafish Model for ML II
- A Novel Use for Acid Ceramidase in Cell-Based Therapies for Degenerative Joint Diseases, including the Mucopolysaccharidoses
- Spatial Navigation and Working Memory Tests Demonstrate Neurological Deficits in a Murine Model of Hunter Disease.
- Suppression of a Nonsense Mutation in a Mouse Model of Hurler Syndrome
- Cloramphenicol: A Pharmacological Chaperone for MPS I?
- Impaired Medullary Haematopoiesis in Murine MPS I
- Genetically Determined Storage of Heparan Sulfate Interacts in GalNAc Transferase Null Mice to Confer and Substantiallyt Accelerated MPSIII Phenotype
- SBC-103, a recombinant Enzyme Replacement Therapy Demonstrates Potential for the Treatment of Sanfilippo B Syndrome
- Development of MPS IVA Syndrome Cellular Models Using Patient Specific Induced Pluripotent Stem Cells
- Gene Expression Profiling of a Mouse Model of Fabry Disease
- Liver-Directed Gene Therapy for Mucopolysaccharidosis Type I
- A Metabolic Study Leads to Detection of Novel Fabry Disease Biomarkers
- A Pilot Program Screening for Fabry, Pompe and MPS I in a Newborn Screening Laboratory: The First 60,000 Samples
- Effects of Minozak and / or Genistein on the Central Nervous System of Sanfilippo Syndrome Type B Mice
- Cardiac Ultrasound Findings in Sanfilippo A Syndrome
- Serial ECG, Echocardiography and Holter Monitoring in Children with Anderson-Fabry Disease
- Mucopolysaccharisis VI: Development of Clinical and Laboratory Guidelines for Diagnosis
- Mosaicism of Podocyte Involvement in Untreated Females with Fabry Disease

WORLD also enabled exposure to the current findings of the following Clinical Trials:

Oral Migalastat HCL (AT1001/ GR181314A) as an Investigational Therapy Evaluated in Females with Fabry Disease

Conclusion: Data suggests that Migalastat is safe and tolerable investigational therapy with emerging pharmacodynamic evidence of efficacy in symptomatic Fabry Disease females with amenable GLA mutations.
Fabry Disease Mutations Addressable with Migalastat HCI, and Investigational Chaperone Therapy. Screening Results from FACETS, a Phase 3 Study in Male and Female Patients
Conclusion: The majority of male and female Fabry Disease subjects screened for FACETS carry missense mutations. Of these subjects, 83% are considered to have addressable mutations and were potentially eligible for the study.

Long Term Outcomes of a Phase 1/2, Multicentre, Open-Label, Dose-Escalation Study to Evaluate the Safety, Tolerability. And Efficacy of BMN 110 in Patients with Mucopolysaccharidosis IVA (Morquio Syndrome Type A)

Conclusion: 20 MPSIVA patients aged between 5-18 years enrolled in the study. They were dosed at 0.1, 1.0 and 2.0 mg/kg/week for 3 consecutive 12-week periods, followed by a 36 – 48 week continuation phase dosed at 1.0 mg/kg/week. 17 patients enrolled in the Phase 1 / 2 extension study dosed at 2.0 mg/kg/week. Overall BMN 110 was well tolerated. Although most patients experienced study drug-related adverse events, most events were mild to moderate and not treatment limiting. One patient experienced a serious reaction on lowest dosage and withdrew from the study and anther could not complete all planned infusions due to infusion associated reactions and discontinued treatment in the continuation phase. Measures of 6-minute walk ditance, 3-minute stair climb and respiratory function generally improved and mean improvements over baseline were sustained at 2 years. Urinary Keratan Sulphate levels decreased, with the lowest levels occurring during dosage at 2.0 mg/kg/week in both the doseescalation phase and extension study. These data support the selection of the 2.0 mg/kg/week dosing regimen in the ongoing Phase 3 randomised, double-blind placebo-controlled trial of BRM 110 in MPS IVA patients.

Enzyme Replacement Therapy for Patients with Alpha-Mannosidosis

Conclusion: Lamazym is a recombinent human alpha-mannosidase, developed for weekly intravenous enzyme replacement therapy. The aims of the A phase I-II study is to evaluate safety and long term efficacy of LAMAZYM treatment. 10 patients were treated. As part of the safety and efficacy evaluation the motor function, cognitive functon, measurement of oligosaccharides in serum, urine and cerebrospinal fluid and CSF-Tau-protein4 were invstigated at baseline, 3 and 6 months. Serum, urine and CSF oligosaccharides decreased, motor function and cognitve function improved. Until now the only safety concern was two patients developed anti-Lamazym-IgE, though no anaphylaxis was observed. These preliminary results suggest that ERT with rhLAMAN is an encouraging new treatment for patients with Alpha Mannosidosis. The study is designed to continue for a total of 1 year where final conclusions can be drawn.

The Princess of Spain, Letizia Ortiz, inaugurated the VII Spanish National Congress of MPS

In her speech, in Catalan and Spanish, the Princess Letizia has advocated philanthropy as a way to promote scientific culture, because, in her words, "all institutions, companies and also any citizen should get involved and be able to say anywhere: I am research". She also also encouraged everyone to visit the website of the Spanish MPS Society, www.mpsesp.org, a group of inherited disorders that affect both adults and children, who have a life expectancy of 10 to 15 years and which today have no curative treatment affecting vital organs.

Attending the Congress, held on the Campus of Bellaterra of the Autonoma University of Barcelona on 1 October 2011, were researchers and physicians, families and affected children with which the Princess was very close, because she knew some of them at the previous Audience that remained with her at the Zarzuela Palace the past January 31, 2011 in Madrid.

In the nearly two hours in which she was in Congress, the Princess Letizia talked to everyone, took photos, and became interested in the activities that were taking place with children in the MPS nursery.

The Minister of Health of the Generalitat of Catalonia, Mr. Boi Ruiz, who also attended the ceremony, recalled that there are 400,000 people who have some of the 7,000 known rare diseases and in Catalonia there is a difficult diagnosis. In his speech he announced that the Catalonian Government has opted that the affected patients and their families don´t have to continue to suffer and end the process of pilgrimage that they suffer before a diagnosis. He explained that it has created a platform and a network to identify who researches in these diseases, how doctors treat to these patients and how many patients there are, to try to join forces and become more effective in the fight against these diseases.

At this congress the researcher Fatima Bosch explained the status of research for a gene therapy against these diseases to be able to directly restore the defective enzyme in the affected organs.

"All institutions, companies and also any citizen should get involved and be able to say anywhere: I am research" said the Princess of Spain.
Jordi and Mercedes Cruz

Challenging Behaviour Foundation

The Challenging Behaviour Foundation new website launch

This year the Challenging Behaviour Foundation (CBF) is celebrating its 15th anniversary. Over the past 15 years we have supported thousands of family carers and professionals, but we know there are many more people we still need to reach with our information. To help achieve this aim we are launching our new website. Families and professionals will be able to access more information online than ever before - new features include an interactive map, video clips and online ordering.

Using our new website and social media as a way to enable families not only from the UK, but also those around the world to access the information and knowledge we have about understanding and managing challenging behaviour is an appropriate tribute to our 15 year anniversary. To view the changes go to www.challengingbehaviour.org.uk The Challenging Behaviour Foundation was founded in 1997 by Vivien Cooper, mother to Daniel, who has a severe learning disability and Cri-du-Chat Syndrome.

"When Daniel started to bang his head on hard objects when he was a year old practical information and support was not readily available, and we struggled as a family to get good advice about how to support him. By the age of nine, Daniel's behaviours were such that we were all struggling to contain them – at home, school and respite - and it was decided that he needed to go to a 52 week specialist residential school. Unfortunately this school was over 270 miles from our home."

As a result of her own experiences with Daniel Vivien has worked to help others access the right kind of information and support to help them understand and manage their family member's behaviour. Working with leading specialists in the field of learning disability to develop resources to support this understanding, and actively encouraging professionals and family carers to work in partnership have led to the CBF being influential in major changes for people with severe learning disabilities and their families.

Winter Newsletter

'Out of sight, out of mind?' the Winter issue of 'Challenge' (newsletter of the Challenging Behaviour Foundation) is now available. This issue focusses on how we support individuals with learning disabilities and mental health problems.

With ten people now having been charged in connection with the ill treatment and neglect of people with learning disabilities following the BBC Panorama programme 'Undercover care: the abuse exposed', 'Out of sight, out of mind?' challenges the reader to keep up the pressure and take action to ensure that care for individuals with complex needs is improved.

'Challenging behaviour and mental health in children and adolescents' highlights the fact that children and young people with learning disabilities are recognised as being at increased risk of mental health or behavioural problems. Other topics include 'Understanding Sectioning' and features families who have experienced sectioning under the Mental Health Act, including situations in which a law designed to protect is sometimes used in ways which are unhelpful.

Regular features include 'your questions', new resources, and 'Comment' by Tony Osgood, Lecturer in Intellectual & Developmental Disability, Tizard Centre, University of Kent.

'Challenge', the newsletter of the Challenging Behaviour Foundation, is produced three times a year and is available free to download from www.challengingbehaviour.org.uk or by emailing your postal address to info@thecbf.org.uk .

Challenging Behaviour Foundation
Email: helen@thecbf.org.uk
www.challengingbehaviour.org.uk

General Enquiries:
Tel. 01634 838739

Family Support Worker:
Tel. 0845 602 7885 (individual telephone support for families at the cost of a local call)

The Challenging Behaviour Foundation is a registered charity (no. 1060714) supporting families caring for individuals with severe learning disabilities.

MPS 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 the **MPS 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...

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 providing 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.

We welcome visits and discussions to explore the different ways in which you can help us improve the lives of individuals with MPS and related diseases, and the families who care for them. We have an effective range of monitoring and evaluation processes in place and will provide regular reports on whatever aspects of our work you chose to support.

Previous grants have enabled us to set up new services, such as our telephone helpline, and to organise events such as regional family days and sibling breaks.

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**.

BBC Children in Need

A grant from BBC Children in Need enabled the MPS Society to run the 2011 Sibling Week, an event which was hugely enjoyed by the group of 8 – 15-year-olds who travelled from all over the UK to attend. It took place at Windmill Hill PGL Activity Centre in East Sussex and included a range of exciting activities for these children, all of whom have one or more brothers or sisters living with an MPS or related disease, or who have lost a sibling to one of these conditions.

We have been very pleased to receive many such grants from BBC Children in Need to run similar events since 1982. Each year we follow a tough application process and have been successful in being approved to receive funding on many occasions over the years. After each event we send detailed reports on how the money has been spent and the benefit provided to the children by the grant. The grant this year meant that we had the necessary funds for this group of young people to spend four nights and five days at the PGL Activity Centre and for two MPS staff, a Trustee and a trained volunteer to accompany them, as well as receiving the support of the PGL staff. These adults were there to assist with all aspects of the holiday, including the health and safety of the group, organising the day-to-day running of the event such as accompanying the children on their activities, supervising them at meal times and being an overnight presence. They were also there to deal with any emotional or practical issues the children might have whilst away from home, and to encourage them to take part and, above all, enjoy themselves!

These residential holidays are so important to these siblings, as it gives them the chance to have a break away from home and to spend time with others in the same position as themselves and share experiences. These children don't often have the opportunity to meet up with others who have a brother or sister with MPS, so they find it really helpful to see that they are not alone, and realise that there are others who share similar feelings. The children grow in self-confidence as they try out new and challenging activities, and have the much-needed opportunity to relax and be themselves. They form lasting friendships and leave with fond memories of a wonderful break.

Without grants such as this one, we would not be able to run these vital events.

D'Oyly Carte Charitable Trust

We were recently delighted to receive a grant from the D'Oyly Carte Charitable Trust for the continuing production of our Children's Newsletter.

This Newsletter is designed specifically for children and younger teenagers affected by MPS and related diseases, including siblings. We piloted this in 2010 and found that our younger members responded very positively to having their own publication. Affected children and their siblings are benefiting from reading about, seeing and writing about all aspects of living with these diseases.

Charity walk raises £3720 for MPS

Ken and Anne Hooper held their biannual charity sponsored fun walk in aid of the MPS Society on Sunday 4 September 2011.

Around 130 people came along to walk the modest 4 miles in the beautiful countryside around Throop and Hurn on the outskirts of Bournemouth. Sadly the walk always takes in the fields along the river Stour where the Red Arrows pilot, Jon Egging, was tragically killed two weekends before the walk.

All those that came had a ploughman's lunch with local Ringwood beer; there was also a book stall and raffle as well as a market stall of local produce.

£3720 was raised for the MPS Society. Ken and Anne have been putting on a charity walk for 16 years (alternate years are in aid of the Royal Bournemouth Hospital GI Cancer Research Fund Charity) and have raised over £50000 during that time.

Haddenham Mummers and Towersey Morris Men fundraise for MPS

The Haddenham Mummers performed their small play 23 times over the Christmas period in various local pubs and restaurants, at the Haddenham Village Carol Service, the Winterfest, the Red Cross lunch and at the New Year's Eve Ceilidh. At each performance they took their customary collection. As a result the Mummers have kindly donated £730 to go towards research into Sanfilippo disease. The District Age Concern were the other charity benefitting from a similar amount. For more information please visit www.bucksinfonet/haddenhammummers

The Towersey Morris Men recently donated £600 to be put towards research into Sanfilippo disease. The Towersey side performed their Mummers' Play over 30 times during the Christmas period and took a collection every time. They shared the takings equally between the MPS Society and the MS Trust, their other chosen charity. Their play differs from the Haddenham one by being topical rather than historical. This year they featured Rupert Murdoch (as the baddie), Angela Merkel as the fixer and Silvio Berlosconi as the doctor. By making him Italian they were able to rhyme tongue with Bunga Bunga!

Friends of Bowen Lodge support our work

The Friends of Bowen Lodge recently nominated the MPS Society to receive £250. Andy Hardy very kindly represented the Society and collected the cheque on our behalf. Andy's son Matthew suffered from Sanfilippo disease. Andy tells us about the evening:

'I arrived at the Aylesbury Masonic Centre to be faced with a sumptuous buffet and a room full of people, none of whom I knew. However, once I declared my name and that I was from the MPS Society I was greeted by one of the Bowen Lodge members and I soon got chatting to people from other charitable organisations. And what a mixed bunch we were! Charities such as MacMillan nurses, Stokenchuch Scouts, Florence Nightingale Hospice, Milton Keynes Young Carers, Alexander Devine Hospice and Help for Heroes and many others were represented. There must have been somewhere between 25-30 different associations.

Everyone was called up in turn and we had to give a five minute presentation on the Society we represented and how the money would be spent. A member from the Lodge or Chapter donating the cheque then came forward and duly handed it over. I was 'second on' so didn't have any time for the nerves to set in!'

Thank you to Andy Hardy for representing the Society on this occasion and to the Friends of Bowen Lodge for their kind support.

Crafts Day in aid of MPS

Dorothy Robinson recently donated £200 to MPS and wrote to tell us about her Beginners Craft Day...

'It all started a couple of months ago. My neighbour Sheila asked if I would be willing to join her and her sister Cherry in holding a beginners Craft Day. She said she hoped she could doante the proceeds to charity. Later she asked if I would like the proceeds for the MPS Society, and of course, I jumped at the idea.

As we were hosting the day for Charity, Sheila persuaded her Church to allow us to use the hall free of charge. My husband, Colin, said he would make pumpkin soup and cakes for refreshments. We held a small raffle, and in doing so were able to obtain funds from people who were not interested in craft.

We were initially disappointed because we only had twelve people who wanted to come to the day, but with hindsight we now realise that this was quite sufficient for us to give all the instruction needed. All the ladies took away with them eight cards they had made and were so delighted with the day that they have asked for another!
Dorothy Robinson,
nanny to Hannah Shannon (MPS III).

Photos below show Grampy Colin serving lunch and Dorothy demonstrating crafts on the far right.

Card party and raffle raise £127

Graham and Margaret Moore held their annual card party and raffle and donated £127 for MPS.

Graham and Margaret are the grandparents of Samantha Brockie who has MPS I. They tell us that Samantha now has her own flat which she shares with her boyfriend. She still goes home for her weekly transfusion because she doesn't have the room to store her equipment but she is very happy and can easily catch the bus at the end of her road. They say it is nice to think that Aldurazyme has enabled Samantha to be independent although she does need help with heavy shopping.

Endurance horse ride for MPS

Timmy Stansfield completed the Mary Towneley loop 47 mile endurance ride on horse back on 19th September 2011 departing from Wardle Reservoir, Rochdale.

He writes 'They raised the money for the MPS Society as Timmy's girlfriend Rebecca Thomson and her sister Adele Wroe both suffer with Fabry Disease. Rebecca has to have the treatment once a fortnight which costs thousands of pounds each time, the disease is very rare and the MPS Society do alot for the sufferers, so we would like to try and do something back.' They raised £241 on their justgiving.com page www.justgiving.com/timmy-stansfield

Amelia's sponsored skydive

Julia and Alex Brown recently wrote to the MPS Society enclosing a donation of £255.

Their letter reads: "This donation is from my granddaughter, Amelia Hill, who did a sponsored parachute jump for her 16th birthday in October. It is in memory of her Aunty Claire Hill who died of Sanfilippo 19 years ago. Sadly since then, her mother Emma Hill died suddenly in December and Amelia is now living with us. There will be another donation to follow in Emma's memory. After Claire died, Emma went to live in Norway to be a nanny for an MPS little boy for Oddrun and Knute Bache. The MPS Society was always very special to us Emma and to us."

MPS Skydiving Team raise £4698 for MPS

Members of the MPS skydiving team 2011 raised a wonderful £4698.39 through Justgiving.com in aid of the MPS Society.

One of the team members is Dan Taylor, father of Cody who has MPS I Hurler disease. We would like to thank Dan and the other members of the team for their support - Davie Perry, Jessica O'Neill, Matthew Barlow, Steve Pritchard, Vicki Perry and Warren Davies. To visit their team page go to www.justgiving.com/teams/mpsskydiving

Bristol Postal Social Club

Julie Kembrey collected a cheque from the Bristol Postal Social Club for £1010. She tells us all about it:

"On Wednesday 25 January 2012 I accompanied my Mum, Marina Foster, to receive this cheque on behalf of the MPS Society. We were invited to attend one of their monthly meetings at Snooker City, Knowle, Bristol. They gave us a very warm and friendly welcome and were interested to hear about the work of the MPS Society.

As you know, Mum runs a very successful charity shop, Marina and Friends, in Sandy Park, Brislington, Bristol. One of the members of the Bristol Postal Social Club, Margaret Fletcher, is a regular customer. She kindly proposed the MPS Society as the Charity of the Year for 2011. Throughout the year the group raised money during their monthly meetings. The fundraising activities included raffles, sales tables and bucket collections."

We would like to thank the Bristol Postal Social Club for their kind donation and Julie Kembrey and Marina Foster for their continued support to the Society.

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**

Great North Run raises £1006

Mark and Debbie Burniston took part in the Great North Run in September 2011 for the third time in memory of Jake Corcoran and raised £1006.30 for the MPS Society.

This year they decided to do something other than asking people to sponsor them. So they decided to hold a charity race night at Woodland Glade Sports Centre at which they raised £715.30. They raised a further £291 in sponsorship as people asked to sponsor them!

They would like to say a great big thank you to all their family, friends and neighbours for their continued support and generosity. A special thanks goes to Anthony and Helen Corcoron (parents of Jake) for all their help, especially on race night.

Gillian and Gemma McCann did a sponsored tandem skydive for MPS and raised £983 including gift aid on their Justgiving.com page.

Paul Moody ran in the BUPA Great Yorkshire Run 2011 and raised £567.50 including gift aid on his Justgiving.com page.
Elliot Moody ran in the BUPA Great Yorkshire Run 2011 and raised £117.50 including gift aid on his Justgiving.com page.

Arron Vandepeer took part in the Virgin London Marathon 2011 and recently raised a further £50 in sponsorship monies.

Amelia Hill took part in a sponsored skydive in October 2011 for her 16th birthday. The skydive was in memory of her aunty, Claire Hill, who died of Sanfilippo disease 19 years ago. Amelia raised £255 from her skydive.

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 Manchester Run	20 May, Manchester city centre, 10km
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
Great North Swim	22/24 June , Windermere
Great London Swim	26 May, London

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

Vietnam Cycle Challenge in memory of Thomas

My daughter Joanna and I decided last year that we wanted to do something special in memory of Thomas, her brother, my son who died of Hurler disease just over twenty years ago when he was only 12 years old, writes Alan Beavan…

We especially wanted to raise money for the two charities that supported our family so much during Thomas's short life so I decided to raise money for The MPS Society www.mpssociety.co.uk and she for Helen House Hospice www.helenhousehospice.co.uk where Thomas had regular respite care. Both organisations were a godsend to our family and we have such good memories of all the support and friends that all of us made, during what was a very difficult time for us all.

My wife Deirdre and I were lucky to have a healthy daughter Joanna before Thomas was born just 18 months later. We were also fortunate to be able to have two further healthy children Samuel and Benjamin thanks to advice and support from the MPS Society and a variety of professionals together, who gave excellent medical care.

As a result of Thomas's illness I decided to change my career from a sales role and move into fund raising. This gave me the opportunity to work for a number of charities during the last 20 years and apart from being a very rewarding career, has given me the opportunity to learn much about fund raising.

I was made redundant last April and this seemed to be the opportunity to do something in memory of Thomas as I decided to become semi - retired, so I had the time.

There are a number of organisations who provide fundraising events for charities both in the UK and overseas and I knew quite a lot about Skyline www.skylineevents.co.uk an events company through my work, having been a participant on a couple of occasions with them.

As Joanna and I both enjoy cycling we decided to choose one of Skyline's overseas challenges and chose to go to Vietnam and attempt the 420 mile mountain challenge.

You may imagine that apart from the cycle training and other things involved with a trip like this that we also had to consider how we would raise the money. Essentially we agreed that we would not do any joint charity fundraising mainly because we live over 100 miles away from one another and we felt it would be less complicated and easier for people to focus on one charity for each of us, especially for family and friends.

Fundraising. First we both set up a justgiving web page www.justgiving.com and then contacted (FROGS!) an easy acronym that I learned from work colleagues, Friends Relations and Organisations Groups and Societies that we knew especially within our own community. We asked for sponsorship and support. Joanna decided to organise one major event with a target to raise £2000 and I decided to do two smaller ones with a joint target of £1000. My first event was a charity lunch tennis match held at my tennis club. This proved to be a fun event and about 30 people attended and £390 was raised. I asked for £10 to enter and the extra amount came from a raffle, all food and drink was donated by members. The second event was a bridge afternoon tea and 64 people attended and this raised £730. We charged £7.50 per person ie £30 per table =£480 .We had some high value raffle prizes given by local business and the raffle raised £180 and also received over £90 in spontaneous donations after the event. To date a total of more than £3600 has been raised on behalf of the MPS Society excluding any direct donations to the charity.

Promotion. Apart from the just giving page Joanna and I had a friend take a photo of us on our bikes and I sent this to my local paper with a brief article and a copy to another local publication. Many of my friends saw the article and this helped with direct cash support and justgiving sponsorship. I did a follow up with photos of the tennis and bridge events to thank everyone and to let them know how much had been raised and included some of our cycle ride.

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 Cycle Challenge. This was brilliant and combined genuine hard work on some days with some great fun. We were a small group of 16 all raising money for various charities and we spent 10 days covering the hilly but glorious terrain in north Vietnam. Much of the trip was in the more remote villages and we were made very welcome by the locals especially the children who waved a smiled at us with glee.

The trip gave Joanna and I the chance not only to do something positive in memory of Thomas but also the chance to talk about him and remember the many fun times we had as a family. It helped us to share what it was like being a sister or a father of a child who required such care and love and to appreciate his impact on our lives. We both agree that Thomas has given us both an appreciation of our own lives and a better understanding of suffering and love. **Alan Beavan**

News

Pair back from Vietnam

Father and daughter complete Asian cycling challenge

By Elinor O'Neill

A FATHER and daughter completed a scenic yet challenging bike ride in Vietnam in memory of a family member who died from a rare hereditary disease.

Thomas Beavan was born with a mucopolysaccharide illness called hurlers disease which left him disabled and shortened his life to just 12 years.

It is 21 years since Thomas died and Alan Beavan, of Nithsdale Avenue, Harborough, took on a 420-mile ride with his daughter Joanna in memory of Thomas.

Mr Beavan and his daughter have now given thanks and praise to the people who sponsored them and supported their fundraising efforts.

Although they had originally hoped to raise £8,500 the figure has far exceeded this and Mr Beavan says this is thanks to the donations of so many people who knew Thomas and their family.

Fantastic

'It was fantastic. It was quite challenging but it was worth it for the views.'
Alan Beavan

So far, they have raised more than £10,000 for The Society for Mucopolysaccharide Diseases which is also known as the MPS Society and Helen House, a hospice that helped with Thomas's care.

Mr Beavan said the bike ride was challenging but a lot of fun and gave him time to spend with his daughter and to appreciate what a 'wonderful woman' she is.

The MPS Society acts as a support network for those affected by the disease and helps promote and support research into mucopolysaccharide and related diseases.

The charity, founded in 1982, was a great help to the family.

Mr Beavan said the group puts families in touch with each other to create a support network and provides families with individuals to support them.

"They were brilliant," he said. "When it was first started we would have days out and meet other children with the same range of diseases, all with a terminal prognosis themselves."

Mr Beavan (67) trained hard for the challenge when he and Joanna (34) were joined by 24 others who all wanted to scale and conquer the hills and valleys of Vietnam on their wheels.

There were Norwegian, Irish and Spanish people taking part as well as people from Loughborough. Although two had to pull out, the rest all completed the challenge.

He said: "We had a wonderful time. The trip was brilliant, it really was fantastic. It was quite challenging, we had a couple of 2,000-metre climbs but it was worth it for the views."

Mr Beavan has raised thousands for various charities over the years including The Children's Society and he has already set his sights set on another fundraiser next year.

To add to the Beavans total, visit website www.justgiving.com/Alan-Beavan or www.justgiving.com/Joanna-Beavan.

Making friends...Alan and Joanna Beavan in Vietnam with local children also on a bike.

Made it...Alan and Joanna with the rest of the party at the end of their Vietnam cycle trek.

Vietnam bike challenge in memory of Thomas

Father and daughter aiming to raise £8,500 for charity

KIBWORTH TENNIS

Charity Tournament 2011

Mongol Rally for MPS

Here is what the organisers of the Mongol Rally have to say about it...

"Imagine you're lost in a massive desert, hundreds of miles from civilisation, driving a car your granny would be embarrassed by. Then all of a sudden all your wheels fall off and the search for tools turns up a dirty sock and two dried apricots. That's the Mongol Rally - 10,000 miles of pure adventure over mountains, deserts and some of the most remote terrain on the planet"

Chris and I couldn't pass up this kind of opportunity. Recently out of college with some time to spare before having to think about jobs and other distractions. We chose a written off Nissan Micra as our trusty steed that would carry us east. A few tins of pasta, jerry cans for water and fuel, a tent and the penguin world map made up our primary equipment.

The following is our collaborative account of the journey as it happened. We started at the Goodwood racetrack. Completed a lap and then we were left to our own devices (no, not sat nav. Cheating!). With any luck, we would literally be in outer Mongolia within a month.

Goodwood, West Sussex, UK - 23rd July 2011: And we're off! Next stop Dover. Already lost, just following another rally car!

Luxembourg - 24th July 2011: Travelling with [teams] "Fear and Alexes" and "Banana Hammock". Broken most of the traffic laws now. Just on our way in to Germany. Czechout [party] here we come!

Kecskemet, Hungary - 27th July 2011: After a brief detour in Slovakia, we're speeding down towards Serbia. Listening to Hungarian classic fm. Fuel prices continue to stay around UK levels and at 80 mph, we're spending about £50 a day! Passed a couple of rally cars so hopefully going the right way! Roads are still perfect and scenery green - expecting this to change in a couple of days...

Istanbul, Turkey - 29th July 2011: Istanbul is big! Bumper to bumper and mirror to mirror with HGVs into the city. Beeping, undertaking and tailgating. We're starting to give as good we get though! Got completely lost on the way in and turned into a side road. The locals instantly took interest and we explained by pointing at the map where we were from and where we were going.

Amasya, Turkey - 30th July 2011: The motel was sooo dirty! Woke up feeling more disgusting than any night camping so far. Still, it was the impetus we needed to get going early. We managed to get out of Istanbul with only a couple of near misses and blasted to the end of the motorway. The road since has been pretty mixed and the local driving style is as erratic as ever. We're back in the mountains and have spent the last 3 hours sitting with a number of Turkish gentlemen in their shack by the side of the road. They seem to buy and sell peaches.

Tabriz, Iran - 1st August 2011: It's been such an up and down day! We managed to find a guy to fix a sump guard to the car and felt great on the way to the Iranian border. We watched in horror as we passed a queue of lorries 3km long waiting to get out of turkey. We parked before the border and did a check for any stray alcohol or other contraband. At this point we were approached by two guys wanting to change money then deal with our V5 form. Passed on both those offers and moved up through the Turkish border. Desperately trying to keep track of all our documents whilst fending off dodgy looking civilians and getting the things we needed, the car was searched, our carnet stamped and visas accepted. We paid 80 dollars for a piece of paper and had to give someone a t-shirt to get out but we think we got off pretty lightly. Once out, we were tired, dehydrated and very shaken. Chris still remained chipper but I was quite low. We needed to see people - safety in numbers. I called Alex and he said they'd reached Tabriz. Chris said he'd drive into the night to get there, braving THE worst driving we've seen - it is so dangerous. Nobody knows how to drive. Against all odds, we've found the hostel in a back street in central Tabriz.

Kaka, Turkmenistan - 5th August 2011: So we pulled up to a service station and got ripped off. Realised immediately but it took us 15 minutes of increasingly heated argument to get back most of what we were owed. We got out onto the road going east. We think the temperature must have been mid 40s and we are literally in desert with nothing as far as the eye can see. The road has warped in the heat to the extent that there are grooves that we have to avoid dragging our sump guard along, bumps and pot holes. I was driving at about 45 along a relatively good stretch but suddenly hit a huge pot hole. I took out the back right wheel. I felt and still feel awful. We got out and struggled to get the jack under the car (this was untested when we left England) then to get it to lift off the ground far enough for the wheel to come off. Eventually we replaced the wheel. A team stopped and hammered out the dent in the rim. We'll try to get a new tyre tomorrow. We've dropped our speed to 35 but at least it's cooler now the sun is going down. We'd heard good things from past rallies and I thought we'd get further before having a serious problem. Still, stiff upper lip.

Turkmenabat, Turkmenistan - 6th and 7th August 2011: Last night we ended up camping just up the road from where we lost the wheel. I pumped some metal through the car's speakers and had a bit of a mosh in the desert - felt much better afterwards! The night, however, was far from pleasant. The temperature stayed in the high 30s all night, rush hour in Turkmenistan is apparently all night so the passing cars and HGVs kept us up. Plus we were by a railway yet again! We left early at about 7am to get to the town of Mary. The first garage we pulled into fixed the busted wheel and pumped the tyre up. It cost 2 pounds. By this point though, I felt like I was about to collapse from heat and dehydration. We found a small shop and bought 15 litres of water to last us the next 2 days. Back on the road and the pot holes were more sparse although Chris had a bit of a bump, we knocked the wheel rim back into shape. We traveled between 30 and 50 mph the rest of the way through sandy desert. Sand obscured the view occasionally but we saw some camels! On the way into Turkmenabat, we passed a team being hassled by 2 policemen and we were flagged down as well. The guys told us they had had to clean their windscreen and the 'arseholes had checked the jerry cans'. They drove off having agreed to meet us at a hotel and we were left to deal with the 'pygg' (Turkmen word for police!). They instantly identified the dent in our door as a reason to fine us 100 dollars. It was pretty evident this was our first bribing opportunity. We offered 20 bucks. They came down to 50. We stuck at 20 but after a couple of minutes they got angry and started asking for 100 again. So we gave them 40 dollars and that seemed to satisfy them. A steep payoff but I was just excited to be involved in such shady business! We found the hotel. It's 17 degrees inside and over 25 outside. Good call. Tomorrow, Uzbekistan border and perhaps Samarkand.

Bukhara Province, Uzbekistan - 7th August 2011: Ok so fuel is tough to get here! Plenty of fuel stations but we found only one selling what we hope is unleaded 95. It took a lot of rather panicked driving around to find it but we think we got some. We took a side road off the motorway and have once again stirred up an entire village. Everyone is really friendly. We're staying in a young chap's house tonight and have been on the phone to all their english speaking friends! "Hello! Nice to meet you!" hope to actually get some sleep at some point. Another big push tomorrow, want to reach Tashkent!

Almaty, Kazakhstan - 11th August 2011: We made it to Almaty! The roads were excellent getting here. We all had quite a lot of police trouble though. We were busy going along the motorway and suddenly hit a police checkpoint with a 20kph speed limit. All three cars were going about 40 by the time the police registered the speed and we all got fined 50 dollars then later on we got caught overtaking in the wrong place. I think all in all we were stopped 4 times. I'm sitting in a hotel which doesn't seem to have changed since it was under soviet control! We're off to a sleazy looking club from the 70s for Alex's party. Heard about the riots [London and Manchester riots] - I thought we were supposed to be in the dangerous countries!

Barnaul, Russia - 16th August 2011: Got through the border no trouble. Tin can, having been in Russia earlier on the rally, were nervous of the country, police and its people. We proceeded under the speed limit to Barnaul. The buildings are stunning here. Modern and well designed. We stopped on a main road. Chatty from tin can, Chris from casbaatah and I went looking for a hotel. After about 30 minutes we came back to the cars to meet all the others running in the opposite direction looking terrified. Some drunkard had punched one of them in the face and kicked another in the stomach. We could see him laying into the cars. He got distracted by his equally drunk friend and we jumped in the cars and drove away. Not a great welcome for us! The hotel was excellent and after a shower, we donned our ragged glad rags and headed for food. Having eaten, most of the guys headed for more at the local subway. Chatty, Jamie and I got talking to some guys on the street. They all had modded cars, looked like something out of the fast and furious. They were really friendly and one of them took Jamie and I at about 100 mph up and down the main street, straight past the police! Too cool. Late start this morning, we've stocked up on food and cash and are ready to head for Mongolia.

Ulaanbaatar, Mongolia - 25th August 2011: We're here! We've done it! The finish line is a dilapidated building in the centre of the city but everyone we met along the way is here. There's beer and burgers. So elated! Our flights are booked for early Monday morning so we'll be back in London about midday. Time to partay! Thanks to all who've followed us and donated! See you all very soon!

The Mongol Rally is an event run by The Adventurists and more information can be found at www.theadventurists.com.

Hugh Osborne

Fundraising Standards Board

The Society for Mucopolysaccharide Diseases is a member of the Fundraising Standards Board (FRSB). The FRSB is a self-regulatory body for UK fundraising organisations, members of which agree to adhere to the highest standards of good practice in their fundraising activities. For further information please visit our website www.mpssociety.co.uk

FRSB give with confidence

WAYS TO SUPPORT US AS AN INDIVIDUAL

Caroline Peach organised a quiz night and donated the proceeds totalling £20.

Irene Roughton donated £150 in support of her friend Kevin Thompson and his son, Tommy, who has MPS.

Shabana Kausar donated £25 as a Christmas gift in the name of her son Shujah Altaf.

Joan Crespin kindly donated £70 towards research into MPS III Sanfilippo disease.

Ellen Graham donated £20 in lieu of sending Christmas cards.

Mr and Mrs C Ball kindly donated 100 Euros after sharing a flight with MPS families on their return from a visit to Father Christmas in Lapland.

Mr Lee Cooper took part in a market research project and asked for his participation incentive from Ci Research Ltd be donated to the MPS Society.

Pat Rowan donated money in memory of her son, Denis, who's birthday is 27 December.

Danielle Warren and her father organised a quiz evening and raffle and raised £565 for MPS. It was such a success they are hoping to make this an annual event.

Ed Hems donated £150 to the MPS Society after hearing about our work through Paul Wilkes. Ed hopes to get his donation matched by his employer UBS.

Lorna and Russell Ellis recently donated £200 to the MPS Society. Their nephew Matthew Wright died in 2012. He had Hunter disease. Russell, Matthew's uncle, was 50 in December and Lorna had some fun pictures of him and his friends made for her by an agency called RBH. The agency did it for her after hearing about Matthew so she promised she would send the proceeds of their 50th birthday picture auction to the MPS Society. This is to say thank you for all the help the Society gave Matthew's mum and dad, Julia and Mark, and his sister Amy. Thank you to the MPS Society and to Deborah Hepburn at RBH.

Dorothy Robinson, Nanny to Hannah Shannon (MPS III), sent in a cheque for £300 to MPS being the proceeds of donations as payment for woodworking done by her husband Colin and the cards that Dorothy herself makes.

Legacy Gift to MPS

Julie Kembrey recently sent us a cheque for £2692.37 from Mrs Iris Cuff (deceased). Julie wrote in her letter "Mrs Iris Cuff was a regular customer of 'Marina and Friends' charity shop, in Sandy Park, Brislington. She made weekly visits and described it as her 'favourite shop'. Over time, a friendship developed between Iris and my Mum, Marina Foster. She greatly respected Mum's tireless work in raising money for research into Sanfilippo disease, following the diagnosis of Mum's only grandchildren, Francesca and Josephine.

One day, Iris told Mum that she was making her Will, and that she would be leaving a donation towards Mum's fundraising efforts. Mum was very touched by this kind thought and provided her with some information about MPS conditions.

Iris died some time ago and when her house was being cleared, some of her possessions were brought into the shop. Then on Monday 30th January, one of the executors of her Will visited mum at her shop. He presented her with this cheque for £2692.37, once again, expressing Iris' wish to support Mum's wonderful work. A very positive way to start the working week!"

Parachute for Free!

If you have ever wanted to do a parachute jump here is your chance. The MPS Society is looking for adventurous volunteers to make a fundraising parachute jump and if you raise enough sponsorship you will get to jump for free!

There are three types of jump available - an accelerated freefall where you can experience the thrill of skydiving solo from up to 12,000 feet, a tandem skydive from 10,000 feet attached to a professional instructor and a static line jump which is performed solo from up to 3,000 feet - and you can jump from any one of over 20 British Parachute Association approved airfields across the UK.

No experience is necessary as all training is given and if you raise from £360 (depending on the type of jump you choose) you will receive your jump for free.

For a full information pack and everything you need to take part in the experience of a life time, please email **fundraising@mpssociety.co.uk** or phone the MPS office on **0845 389 9901.**

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.

Aegis London donated £250 on behalf of one of their employees Simon Cheal whose cousin suffers from MPS.

Gayle Bradshaw's employer, **Phones4u Ltd**, ran a raffle and donated £200 to MPS. Thank you to everyone who supported the Society by purchasing a ticket.

MPS received £250 from **HSBC in the Community** following Chris Taylor's Jogle for Joseph challenge.

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**.

WAYS TO SUPPORT US AT SCHOOL

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

St Theresa's Primary School

recently raised £185 for the MPS Society. Gracie Mellalieu's mother, Yvette, works at the school. Gracie, aged 5 years, has Morquio.

Yvette also sent us a cheque for £50 raised by the 4th Chester St Mary's Brownies for MPS.

Terry School of Dance show

The Terry School donated £660 being the proceeds from their bi-annual ballet school show held at Gryffe High School Theatre, Houston, on 18-19 November 2011.

Teresa Ferguson, Principal, writes: "140 pupils of the ballet school, from 4 years to adults - from Bridge of Weir, Houston and Kilmacolm - danced in the show, performing 'The Wishing Well' and 'Sleeping Beauty'.

I have chosen the MPS Society because one of my first pupils at the ballet school in Bridge of Weir was Joanne Evans, who suffers from Morquio disease. Joanne took great delight in her dancing while she was able and danced in our first two shows in 1996 and 1997. Sadly Joanne, (now 25 years old) is now confined to a wheelchair, but we maintain contact with her and her Mum, Judy - who is on the board of Trustees for the MPS Society. Judy was an essential part of the performances as she administered all ticket sales and helped front of house.

Parents and pupils enjoyed the performances and were extremely generous in their support. I hope that we will be able to contribute to your funds again in the future."

The grandson of a member of staff at **Sandford CE VC Middle School** has recently been diagnosed with MPS II, Hunter Disease. The school held a balloon race and a gentleman who found one of the balloons kindly sent in a donation to the school to pass on to a charity of their choice. The school chose the MPS Society. We would like to thank the school and also **Dr P J Matthews** who found the balloon.

Staff at **Banff Primary School** donated £50 in lieu of sending Christmas cards to each other in memory of a former pupil.

Farmor's School in Gloucestershire have held various fundraising activities and raised £399.46 to go towards the MPS Society's Project Sanfilippo.

Cheque presentations

The MPS Society is very grateful to our fundraisers and supporters for all their hard work in raising money through organised fundraising events, sponsored challenges and other activites, big or small.

We get a number of requests to attend cheque presentations or give talks on our work. We always like to do these when possible but to minimise the costs to our charity, try to coincide these with other visits in the local area or en route to other meetings or events. Thank you to all our fundraisers for their continued and very vital support. We need you!

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

Mission Fish and ebay for Charity

Background: We aim to help charities get the most out of online commerce.

We want to make it possible for any charity to benefit from any gift from any donor. To achieve that vision we're trying to make it easier to give as a part of daily life online, and working to change the way people think about philanthropy. We are a registered charity (No 1110538).

Our major initiative is **eBay for Charity**, a unique programme that helps charities raise money on eBay. eBay for Charity helps charities of all sizes to raise funds by trading on the eBay marketplace.

The MPS Society is registered with Mission Fish eBay for Charity.

eBay sellers can donate 10%-100% to the MPS Society whenever they sell an item (there is a £1 minimum donation per listing). Pick the MPS Society as your favourite charity to see when you are buying and selling on eBay. You can make this selection on the 'My Favourite Charities' page in your eBay Donation Account.

To learn more about how you can support the MPS Society on eBay, visit *www.ebay.co.uk/charity*

ebay for Charity

Between November 2011 and February 2012 the MPS Society received £69.72 from Mission Fish eBay for Charity.

Any individual leaving 10% or more to charity will have their inheritance tax reduced from 40% to 36% from 6 April 2012. The relief is designed so that the benefit of the tax saving is reflected in the bequests received by charities and not in payments to other beneficiaries. The Chancellor said in his speech 'we want giving 10% to charity in legacies to become the norm'.

Please donate

online: www.mpssociety.co.uk

phone: 0845 389 9901

post: MPS House, Repton Place, White Lion Road, Amersham, Bucks, HP7 9LP

IDEA: Creating a *Justgiving.com* page in memory of a loved one is a powerful way for friends and family to honour them by donating to a cause they cared about. Create your page in aid of the MPS Society, you can write a personal message, add your favourite photos and invite people to make a donation.

www.justgiving.com/sfmd/remember

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**

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.

giftaid it

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...

Topnoth Health Club in Chesham held a bookswap raising £31.50 for MPS. Thank you to the Club's members.

Timsbury Wine Circle donated £22 in lieu of sending Christmas cards.

Sudbury Methodist Badminton Club donated £25 in lieu of sending Christmas cards.

Joanne Haines donated £150 being the proceeds of a collection in memory of her son Lewis.

Lucy Brock's friend, **Sam Coates**, has been holding coffee mornings and face painting to raise money and awareness of MPS and that Lucy's daughter Hannah goes through having MPS.

Louise Lucas and the **Hampden Arms** in Great Hampden Village, Buckinghamshire, held a Boxing Day quiz and raised £155 for MPS.

Sandra Silcock donated £41 being the proceeds of money collected in her silver MPS piggy bank. This money is to go towards research into Sanfilippo disease.

Ann Parsons at Asda Eastbourne who has donated £205 and £63 from the sale of trolley key rings.

All Saints Church in Belvedere, Kent donated £106 in respect of collections from the congregation.

Kim Mills sent in £100 being money donated by friends and family in memory of her son, Andrew Parker, who had Sanfilippo B. It was the 9th anniversary of Andrew's death on 23 December 2011. This donation is to go towards research into MPS III.

Marilyn Eggleton donated £40 in memory of her daughter Kim who would have been 40 years old this year.

Christine Lavery, the MPS Society's Chief Executive, recently celebrated her 60th birthday. In lieu of presents, Christine asked for donations to the MPS Society. We received £810 including a donation of £500 from Christine's brother Terry.

The MPS Society recently received £300 being the proceeds of money collected **in memory of Grant Sim** who suffered from MPS III.

Frank and Eileen Homer, grandparents of Alex Dearn (MPS I), recently celebrated their Golden Wedding Anniversary. Instead of receiving presents, they asked for donations to the MPS Society. MPS received £120.

Lynn Longhorn donated £100 to the MPS Society being the proceeds of a Christmas Fair at her home on 5 November. This donation is to go towards research into Sanfilippo disease.

Anna Eaton donated £30 to the MPS Society being money collected from friends at a recent family party.

Donna Bown donated £20 to MPS being the proceeds of a table top sale at Asda Eastbourne.

Sarah Holland donated £85 being the proceeds from the sale of MPS merchandise amongst her family, friends and local community.
The congregation of **St Andrew's Church, Biggleswade,** donated £523.42 being the proceeds from collections over the Christmas period.

Karen and Andrew Weedall donated £32 to MPS being the commission from a Christmas Webb Ivory order.

'Friends of the Prince of Wales' raised £50 for MPS being the proceeds of a raffle they held over the Christmas period. We would like to thank Ann and Rick Coleman, friends of Dave and Laura Brodie, whose son Will has MPS II Hunters.

Andrew Jones donated £800 being the proceeds of his participation in The Slugger Run 2011 in memory of Gareth Evans.

Rachel Hodgetts ran the BUPA Great Birmingham Run 2011 for MPS and raised £70.75 on her Justgiving.com page.

Anna Bovey and Claire Thomas took part in Run to the Beat 2011 and raised £1181.25 including gift aid on their Justgiving.com page.

Great North Run for William and MPS: BUPA Great North Run 2011
Tom Flowerdew, Andrew Reeves, Sue Flowerdew, Christine Turner, Lesley Bastow, Vic Turner, Ella Turner and Georgina Turner ran as a team to raise money for the MPS Society. They raised **£4700** including offline donations and gift aid on their justgiving page www.justgiving.com/gnrforwilliam

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.

SPECIAL THANKS TO…

Donations

Mrs D Kelly; Margaret Taylor; Emily Mason; Norman Saville; Kate Ferrier; Linda Pack; Jonathan Moore; Mrs J Quant; Wendy McGinn; Linda Collett; Ray Webber; Edward Hems; Kim Dinham-Perens; Marjorie Banks; Valerie Challen; Brian D Souza; Mrs M Griggs; Sue and Vic Lowry; Mr and Mrs J R Bradshaw; Mr T P Watts; Mrs D Duckett; Peter Gordon; FG Robinson; Diane Peirson; John Langford Stacey; J C Womack; Mrs U M Wright & Mr B D Wright; Carol Copsey; Mrs A Baker; Zoe Warren; Lynda Gilmour; David Tonge; Frances Shah; Kathryn Wallis; Barbara Flaig; Marilyn Eggleton; M P Nadin; Michael and Sandra Burke; Kate Tate; Hilary Parker; Ella Turner; Rev. A Stratta; J Banks; Mark Dean; Mr J Scott; Tywyn Women's Institute; Barry Simner; J. Langford Stacey; Neil and Chris Cooper; Wilma Robins; Neverland; Bristol Textile Recyclers Ltd.; Mrs P Todd

In memory

Lewis Browning; Lewis Cato; Graham Sampson; Denis Rowan; Daniel Allen; Gethin Robins; Peter Robins; Sheila Quant

Collection boxes

N.C. & B Lunt Pharmacy; Warren Farm Tearooms; Joan Crespin; Janis Clayton (Shire); Val Evans

Stamps, foreign coins, mobile phones, ink cartridges, jewellery:

Helsby Hillside Primary School; Donna Bown; Karen Robinson; Langlea House Care Home; Ben and Hannah Brock; Sally Cartwright; G Plummer; S Swayne; Ken and Pam Ballard; Shire HGT; Rachel Todd; Mrs M Davison; Sue Lowry; Mr Stone

The Society would like to thank the following donors for their regular contributions by either Standing Order or Give As You Earn:

L Wood; P J Martin; G Stepney; Mr & Mrs Cock; A Dickerson; D Palmer; JE & VR Hastings; E M Lee; R Dunn; S Littledyke; C Gibbs; D & S Peach; I Pearson; J Ellis; Raymond Arnold; N Cadman; J York; Molly Rigby; M Wood; A Tresidder; E Cox; C Lunnon;' K Robinson; Mr Thompson; K Osborne; S Hill; M J Peach; J Wilson; A Sullivan; A Byrne; A Weston; Evelyn White; C L Hume;P Summerton; M Kalsi; Michael Reeves; L Wood; R W Gregory; R Taylor; K Brown; S Home; V Little; G Simpson; W G Cavanagh; M Malcolm; E Mee; M Fullalove; M McCann; R Parkinson; J Dalligan; S Bhachu; C Cullen; P J Martin

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!

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: .....

..... Postcode: .....

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

WANTED!

Adventurous people
throughout the UK
to do something like this...

for

FREE!

Minimum sponsorship required

Help us raise funds by making an exhilarating 10,000 feet freefall parachute jump. No experience is required and if you raise the minimum amount of sponsorship you will get to jump for FREE!

Phone **0845 389 9901**
or email **fundraising@mpssociety.co.uk**