THE MPS MAGAZINE

Society for Mucopolysaccharide Diseases

Support Research Awareness

Spring 2016

www.mpssociety.org.uk

Vimizim Managed Access Agreement

The Vimizim Managed Access Agreement has been given the go ahead by NHS England. Find out more information on criteria and expectations of the Managed Access Programme on pages 30-31

Multidisciplinary Clinics

Great Ormond Street Hospital explain the benefits of holding multidisciplinary clinics for complex conditions, such as MPS, and what happens at clinic.

See pages 14-15

MPS Awareness Day 2016

On May 15th 2016 we will be celebrating MPS Awareness Day and we invite you all to help us get our voices heard!

To find out how you can get involved turn to pages 38-40

Front cover photo: Sally Mitcham sits with her son, Danny, while he receives ERT

Society for Mucopolysaccharide Diseases

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To submit content email;
magazine@mpssociety.org.uk

The articles in this magazine do not necessarily reflect the opinions of the MPS Society or its Management Committee.

The MPS Society reserves the right to edit content as necessary. Products advertised in this magazine are not necessarily endorsed by the Society.

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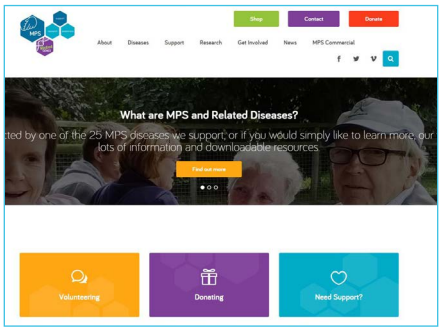

New Website

We are pleased to announce that our website has now been updated with a new design and a clearer navigation system, which we hope will make the site more accessible and attractive to visitors.

Thank you to all the families who so kindly let us use their photos to make the site come alive.

If you have any comments or ideas about the new website, please email info@mpssociety.org.uk. We would love to hear from you!

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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.

Our Aims:

To act as a support network for those affected by MPS and Related Diseases.
To promote and support research into MPS and Related Diseases.
To bring about more public awareness of MPS and Related Diseases

Our Vision

A world that is free from MPS and related diseases.

Our Mission

All children and adults affected by these diseases:
• Can be treated;
• Have equitable access to state-of-the-art clinical management and therapy
• Are active members of society reaching their full potential
• See pioneering gene therapy research offer a cure for these devastating diseases

MPS and 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, neurological deterioration can result in death in childhood.

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

Contents

facebook.com/mpssociety

twitter.com/MPSSocietyUK

Welcome

Welcome to the Spring 2016 edition of our MPS Magazine.

We have got some lovely contributions from our members who have written in with stories of accomplishments and even a poem. You can read these pieces in our 'Your Stories' section starting on page 19. If you would like to share your story in a future edition, please email magazine@mpssociety.org.uk.

We also have the usual mix of up to date information in our 'Research & Treatments' and 'Information & Resources' sections, with something of interest to both professionals and families.

MPS Awareness Day (15th May) is almost upon us and with that in mind we have featured some information on how you can get involved in this important day. Don't forget to join us on Facebook and Twitter to celebrate the day!

Best wishes

The MPS Society

Visit our online shop

www.mpssociety.org.uk.

Purchase our information resources and MPS merchandise including our T-shirt!

Group Chief Executive's Report

Christine Lavery

With the daffodils in full bloom here in the South of England it is a poignant reminder that we are three months into the year and already the MPS team are at full stretch delivering all our members and their families a really busy support programme. In January we hosted a very well attended 'Welsh Information Day' in Cardiff where the elephant in the room was again raised – access to new therapies for the Lysosomal Storage Diseases. However as I write we understand that Vimizim has now been approved for reimbursement for MPSIVA in Wales.

It was a disappointment that the take up for the Scottish Information Day was so poor that we had no choice but to cancel the event. Over the coming months the Advocacy Team will be consulting with our Scottish members and their families to understand the events they most want to see; their frequency and location.

Over the past 12 months nearly two hundred individuals and their families affected by Fabry, MPSII, MPSIIIB and MPSIVA have participated in Patient Reported Outcomes (PRO) surveys. All your anonymised collective contributions have been incredibly valuable in helping regulators, pharma industry and clinicians to improve their understanding of the disease impact and treatment experiences. We recognise that these telephone surveys take time but want to encourage our members to continue to support these initiatives providing vital information only you can share. Over the coming months we hope that our abstracts on learnings derived from these surveys submitted to the Scientific Committee for the International MPS Symposium in Bonn in July will be accepted and shared with the wider MPS Committee. We are looking to the Society for the Study of Inborn Errors (SSIEM) meeting in September to submit our Fabry abstracts. In the 'Research & Treatments' section of this magazine you will see the collective findings from the Fabry Patient Reported Outcomes (PROS), in which 174 of our Fabry members took part. You will also find the abstract on the experiences and information requirements of women with MPS and related conditions during pregnancy and the post-natal period in this section.

After playing a significant role in convincing NICE that patients with MPSIVA (Morquio) should benefit from reimbursed Enzyme Replacement Therapy we are now actively involved again with the NICE process to endeavour to secure reimbursed Enzyme Replacement Therapy for Lysosomal Acid Lipase Diseases (LAL D) and Migalastat an oral treatment for Fabry disease. These NICE evaluations require considerable preparation by the MPS Society writing the submissions, responding to the NICE consultations as well as finding expert patients or parents to write their own submissions and participate in the NICE evaluation meetings. Patient experiences are critical to the process so again we thank the members who have come forward to support our efforts at NICE.

I opened my report referring to daffodils but already the MPS Events team are thinking more about Father Christmas. Following the success of the 2011 visit to Lapland in Finland, at their last Board of Trustees meeting in February, the Trustees agreed part funding for the MPS Society to support another visit to Lapland. We are very fortunate that matched funding has been granted by the Gosling Foundation for the Lapland visit so please look out for the application form in this edition of the MPS Magazine if you want to apply to be part of the MPS Society's truly 'magical' visit to Father Christmas in Lapland.

Christine Lavery
Group Chief Executive

News From the Board of Trustees

The Society's Trustees meet regularly. Here is the summary of the main matters discussed and agreed at the Trustee Board Meeting on 28 November 2015 at MPS House, Amersham.

Governance

The Chairman, Susan Peach advised Trustees that she had now reviewed the responses received in respect of the individual Trustee Activity Appraisal. Key learnings were that some Trustees undersell themselves if they complete tasks within their area of responsibility.

The Group Chief Executive confirmed that through the MPS Magazine the MPS Society has advertised for new Trustees with skills in accountancy and HR but to date has not had any response from the membership.

Financial Management

The reports were circulated previously. The Treasurer presented the figures to 26 November 2015 and reviewed the consolidated budget for 2015. The consolidated budget for 2016 was considered and approved following reassurance that the Welsh Information Day will come in on budget. An updated Reserves policy was approved. A review of the Financial Controls policy was deferred until the February 2016 Board of Trustees meeting.

Risk Management

Following a discussion on the new IT service provider it was agreed Trustee, Tim Summerton and Group Finance Officer, Gina Smith and the new IT provider meet to discuss the risks of using Office 365.

Policy Strategy

Trustee Jessica Reid confirmed she had spent a day with PA to Group Chief Executive in HR and policies, Karen Minashi. Jessica Reid advised Trustees that the number of policies could be reduced by amalgamating policies related to 'leave' and 'conduct' for example.

Personnel

All Trustees confirmed they had received the personnel report. The Group Chief Executive told Trustees that the employees said a fond farewell to Liz Rodda who retired on the 27 November 2015. The Group Chief Executive advised the Board of Trustees that following a consultation with her PA, Toni Ellerton and the Board of the Fabry International Network (FIN) Toni Ellerton has taken the part-time role of FIN coordinator for a trial period.

MPS Commercial

MPS Trustee and Chair/Director of MPS Commercial, Bob Stevens advised the Board of Trustees that the MPS Commercial Board of Directors had met on the 27 November 2015. Bob Stevens gave an overview of the clinical trials and current cash-flow strategy.

Advocacy Support

Trustees confirmed the Support and Advocacy report had been read. The Group CEO confirmed that the two family days in Manchester and Liverpool are taking place this weekend with a considerable number of bookings for both events.

What's On

MPS Regional Clinics 2016

MPSI - GOSH
12th April • 26th July

MPSIII - GOSH
28th June • 13th September

MPSIV - GOSH
12th July • 8th November

Fabry - GOSH
26th April

MPS - BCH
10th June • 16th September
25th November

MPS Transition clinic - BCH
25th April

MPSI Post HSCT (over 6 years) - RMCH
6th May • 1st July • 7th October

MPSI Post HSCT (under 6 years) - RMCH
8th April • 8th July • 14th October

Fabry - BCH
27th May • 28th October

Adult Fabry - QE, Birmingham
12th April • 10th May • 14th June
12th July • 9th August
13th September • 11th October
8th November • 13th December

Conferences and Regional Events

Expert & Patient Meeting on Mucolipidosis - Hilton, Northampton
9th - 10th April 2016

Scottish Get Together - Hilton, Edinburgh
tbc

MPS Awareness Day
15th May 2016

Gulliver's Theme Park Family Day - Milton Keynes
15th May 2016

All Ireland Conference - Hilton Hotel, Templepatrick
20th - 22nd May 2016

14th International Symposium on MPS & Related Diseases - Bonn, Germany
14th - 17th July 2016

Childhood Wood Planting - Sherwood Pines
16th October 2016

Lapland Family Trip - Finland
2nd-5th December 2016

SAVE THE DATE
MPS Weekend Conference 2017 - Hilton, Coventry
7th July - 9th July 2017

Announcements

New Faces

Louise Cleary
Advocacy Support Officer

Hi, my name is Louise Cleary, I am the new member of the advocacy support team, and I will be supporting children and adults that have MPS III (Sanfilippo), LAL D and Gangliosidosis. I started off my career as a nursery nurse and enjoyed working in this field for four years, before I moved into the world of social care. I worked for a local authority for several years as a social work assistant, before studying and qualifying as a social worker whilst working for the same local authority. During my social work training I completed a placement with a Children with Disabilities Unit, and enjoyed the experience of being able to offer families a different level of support. It never ceased to amaze me how much passion and care families would give to their children, even through the tough times and I knew at that point that I would want to return to working with families that have children with disabilities. My passion during work is to support families to the best of my ability.

I am recently married, and I have one cat who I see as my surrogate child. I love to care for those that are close to me, which often is presented in the form of cooking and baking.

Helen Crawley
Fundraising &
Information Officer

I joined the MPS Society at the end of February as a Fundraising & Information Officer. I'm so pleased to be working for a charity with a long history of outstanding achievements and I hope I can continue to help make a difference here.

I came from a background in publishing, marketing and design and have previously worked for a charity producing educational research reports and products. I was drawn to the MPS Society as it seems to have accomplished so much despite being a small charity and really cares about the people it supports. That is made even clearer when you share an office with a dedicated support team and a bunch of enthusiastic fundraisers.

So far I have been writing thank you letters (some of the things you are doing to raise money for MPS sound so much fun!), looking at ways to create more of a buzz around our MPS Awareness Day on 15 May and writing applications for charity of the year partnerships.

When I'm not writing thank you letters and looking for ways to raise money for the MPS Society, you'll find me looking after my 5 year old twin boys or playing cricket for my local women's team!

If you have any great ideas for our MPS Awareness Day or know of a charity of the year scheme we can get involved with let me know at h.crawley@mpssociety.org.uk. Thanks!

Pauline Walker
Finance Assistant to MPS Commercial

I joined MPS Commercial in February as a part-time accounts assistant. Prior to joining, I had not heard of the MPS diseases, but am looking forward to finding out more about the Society and the work it does.

I enjoy spending time with friends and family. I live with my husband Chris, and we have three children and three lovely grandchildren who we see often.

Alex Morrison
Managed Access Programme Support Officer

Hi I'm Alex and I joined the MPS Commercial team at MPS House in January 2016 to support the newly introduced Managed Access Programme for Vimizim. Some of you will have spoken to me already as I have been conducting the quality of life questionnaires. I've also been very busy with setting up the systems we need to record and store all the information collected.

My background is in Medical Communications and I have worked in the pharmaceutical industry and as a freelancer. I have also worked with children with special educational needs and physical disabilities in a large secondary school. I'm working part-time in a job share with Jackie Adam and in my spare time I still do a little freelancing work. I also enjoy being outdoors, in the garden growing vegetables in the summer and walking and cycling all year round.

The team here have been so welcoming and I am learning new things every day. I am thoroughly enjoying my new role and am looking forward to meeting some of our members and their families in the future.

Jacqueline Adam
Managed Access Programme Support Officer

Hi, my name is Jackie and I recently joined the MPS Commercial team as the Vimizim Managed Access Programme (MAP) Support Officer, for those with MPS IVA, Morquio. I will be job-sharing with Alex and responsible for collecting the quality of life data from patients on the programme.

Before starting at MPS House I worked for a number of years as a Medical Writer for a medical communications agency and as a Science Writer at The Wellcome Trust, a biomedical science charity. During this time I wrote about the findings from clinical trials, so this role will be slightly different for me in that I will be the one gathering the data.

It has been a busy few weeks since I joined the team familiarising myself with the MAP and some of the other trials the MPS Society is involved with. I look forward to getting to know those of you who are already enrolled on the MAP during your 4, 8 and 12 months interviews and future MAP participants!

Advocacy

The MPS Advocacy Support Service has been established since the Society was founded in 1982. At this time there were only 40 known families throughout the UK. The support provided was on a voluntary basis and depended heavily on individuals and parents to provide support to individuals diagnosed within their immediate and surrounding areas.

However in 1991, the Society opened its first office and with this the advocacy service we know today was born.

The MPS Society provides, through a team of skilled staff, an individual advocacy support service to its members. The service is flexible and a wide range of support is offered on a needs led basis.

The rarity of these conditions means that in many cases, accurate assessments, support and advice are not given due to the vast majority of social care and health professionals knowing very little if anything about the diseases.

Support provided by the team

• Telephone Helpline

0345 389 9901– the Society provides an active listening service, information and support. This includes an out of hours service

• Disability Benefits –

In understanding the complexities and difficulties individuals and families have in completing claim forms for Personal Independent Payment, the Society continues to provide help and support in completing these forms and, where needed, will take a representative role in appeals and tribunals

• Housing and equipment

– The Society continues

To take a major role in supporting and advocating appropriate housing and home adaptations to enable the needs of an individual with an MPS or related disease to be met. Where requested, we can provide comprehensive and detailed housing reports based on individual need

• Education –

The Society helps members to access appropriate education and adequate provision for its implementation. This is achieved through providing educational reports used to help inform and educate professionals, and in many instances, to inform Statements of Special Educational Need. Where requested, we also provide information days/ talks to schools and relevant professionals

• Respite Care –

The Society continues to work closely with a number of respite providers and, where appropriate can make individual referrals

• Independent Living/ transition –

The Society provides advice, information and support through the transition from child to adult services. This could include access to independent living, learning to drive, further education and employment

• MPS Careplans –

The Society undertakes a comprehensive assessment of the issues which need to be addressed when caring and providing support to a specific individual diagnosed with an MPS or related disease, as well as other family members through the writing of a careplan

• Befriender Service –

The Society links individuals and families affected by MPS and related diseases for mutual benefit and support

• Bereavement support -

For more information on any of the above or if there is anything else that you would like to chat with the advocacy team about please contact us:

Email: advocacy@mpssociety.org.uk
Telephone: 0345 389 9901

Advocacy Resources

The Advocacy Team have also developed a range of information resources focussing on particular issues which are available to download free of charge from the MPS website, www.mpssociety.org.uk

- Life Insurance
- Travel Insurance
- Hospital Travel Costs
- Disabled Access Holidays • Carers Legal Rights
- Carers Allowance
- Wheelchairs and Flights • Guide to Housing and Disabilities Facilities Grant
- Benefits including, Personal Independent Payment, Benefit Cap, Council Tax Benefit and Universal Credit

Each of our England based Advocacy Officers works with specific disease groups as listed. However, every member of the Advocacy Team has knowledge of all the diseases and may at times provide support in other areas dependant on need and individual assessment.

Our Advocacy Support Team

The rarity of MPS and related conditions means that in many cases, accurate assessments, support and advice are not given and individual need is neglected or undermined by policies and practices which, do not address the multi- systemic nature of these diseases.

The MPS Society provides, through a team of skilled staff, an individual advocacy support service to its members. The service is flexible and a wide range of support is offered on a needs led basis. This is achieved through direct contact with our members either in person by telephone, email or letter.

The Society currently has approximately 1200 registered members approximately 750 of these are living with an MPS or related disease. A review of current caseloads over a 12 month period (Jan – Dec 2015) concluded active cases averaging at approximately 150 a month.

The team provide professional advocacy that encompasses support, information, representation with the aim of empowering and promoting access to services and enable our members to express needs and choices.

The advocacy service provides support across the UK and although its main base is situated in Amersham Buckinghamshire we do have a regional service which is delivered in Northern Ireland.

In order to meet the aims and core principles of the service, each advocacy officer must work to a high level of professionalism. In order to do this the following skills, knowledge and person qualities must be present, applied and reviewed regularly

Current staff team professional specifications
- Qualified Social work
- Public / statutory services
- Genetic services

SOPHIE

Manages the MPS Advocacy Team

DEBBIE

MPSIVA Morquio, MPSI Hurler BMT, Hurler Scheie, Scheie, MPSVI Maroteaux-Lamy, MSD, MLII

REBECCA

Fabry
MPSII Hunter
MLIII / MLIV
Mannosidosis, Fucosidosis

LOUISE

MPSIII Sanfilippo type A, B, C and D
LAL D
Gangliosidosis

ALISON

Supports members living in Ireland

STEVE

MPSIII Sanfilippo type A,B, C and D, MLD AGU, Winchester
Geleo Physic Dysplasia
Sly, Gangliosidosis, Sialic Acid Disease

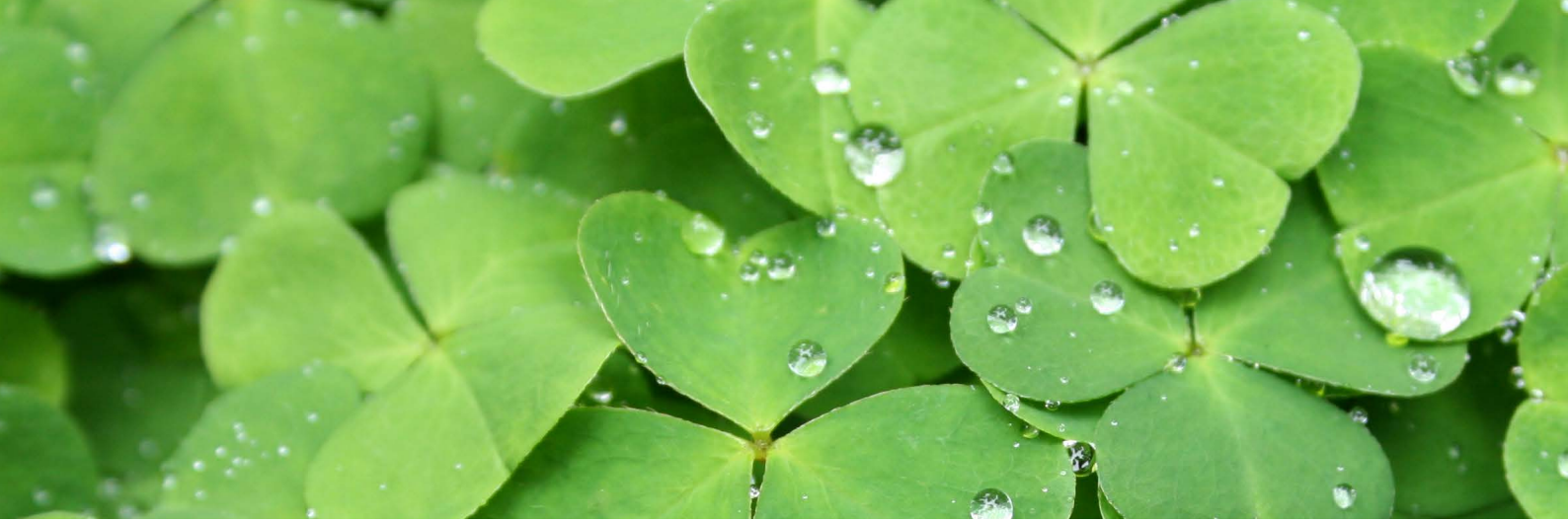
New Members:

Michael has recently been in contact with the Society. He has a diagnosis of Fabry disease. Michael adds, "I have a partner and two boys that stop me thinking about my disease."

Claire has recently been in contact with the Society. She and her son have been diagnosed with Fabry disease. The family live in the Middlesex area.

Bruce and Nicola have recently been in contact with the Society. Their son Jack has a diagnosis of MLII, I Cell disease. Jack is 10 months old. The family live in Aberdeenshire, Scotland.

Mr and Mrs Ali have recently been in contact with the Society. Their sons have a diagnosis of Hurler Scheie disease. Zamin is 5 years old and Qasid is 2 years old. The family live in the North West of England.



All Ireland Advocacy Support Update

The beginning of 2016 has been busy as usual for the All Ireland Advocacy and Support Service. I've been busy meeting families at clinics, visiting schools, attending housing meetings and spending time chatting over a cup of tea with many of you who just needed a little bit of advice and support.

Please remember, if you have any unmet support needs and live in Ireland (North or South) you are more than welcome to contact me on 0044 77862 58336 or a.wilson@mpssociety.org.uk

After our November MPS clinic we very sadly said farewell to Dr Bronagh Sweeney – one of our paediatric doctors in Belfast. Bronagh has relocated with her young family and unfortunately that has also meant a move away from the Children's Hospital in Belfast. Lots of our families have asked me to pass on messages of thanks to Bronagh in the last few months so I thought we should recognise her here and say a big 'THANK YOU' for all her hard work and dedication to MPS families in Northern Ireland. Bronagh has promised not to disappear and looks forward to attending conferences and events in the future so I'm sure you will have a chance to catch up with her soon.

MPSI Meeting Dublin

On the 10th March Christine Lavery, Sophie Thomas and I attended an Irish MPS Society led meeting on Haematopoietic Stem Cell Transplant Outcomes for MPSI. This was a very interesting meeting with speakers from America, England and Ireland covering a range of topics from ERT to neuroimaging.

We heard from Dr Simon Jones and Prof Rob Wynn (Manchester) about ERT and transplants for MPSI and were delighted to see data presented by Christine Lavery that proves just how far things have come since the first transplant for MPSI in the 1980s. The keynote speakers for the day were Prof Paul Orchard and Prof Elsa Shapiro who had travelled all the way from Minnesota and Portland Oregon. We were delighted to hear from Prof Orchard about his experiences of treatment for MPSI and from Prof Shapiro about her research into the behavioural and intellectual outcomes for individuals affected by MPSI.

The day was rounded up by a series of local speakers (Dr Cox, Dr McGovern and Dr Brasnahan) who spoke about the importance of multidisciplinary follow-up from the perspective of a dental surgeon, ophthalmologist and respiratory physician.

The meeting provided a very comprehensive overview of MPSI past, present and future and provoked lots of discussion amongst attendees.

It was lovely to see some of the Southern Irish families as well as lots of local professionals who have a special interest in the MPS Conditions.

All Ireland Conference

2016 is the year of our All Ireland Conference in The Hilton Hotel in Templepatrick and you will already have received your conference programme and booking form. The conference this year is on the 20th-22nd May and we have a packed programme of both local and national speakers. As usual there is a mix of family and professional speakers so there will be something for everyone.

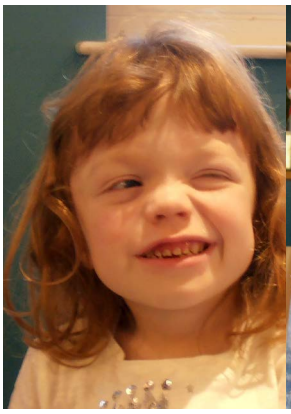
We would be delighted to see you there and by the time you receive this magazine you will be just on time for a last minute registration.

If you have any questions or would like to find out more please don't hesitate to pick up the phone to the Events Team at MPS House on 0345 3899901.

Alison Wilson
Advocacy Support Officer

Clinics

GOSH MPSI clinic - 12th January 2016



Sienna

Toby & Oliver

Once again it has been a busy time for clinics at all the children's hospitals. It is lovely to see families and to catch with the children and see what they have been up to. Clinics are also a good way for the advocacy team to meet new families and to introduce the MPS Society and the work that we do and it's always good to put a face to a name!

Debbie Cavell
Advocacy Support Officer

RMCH MPSI over 6 clinic - 22nd January 2016

Jake

Leighton

Lyla

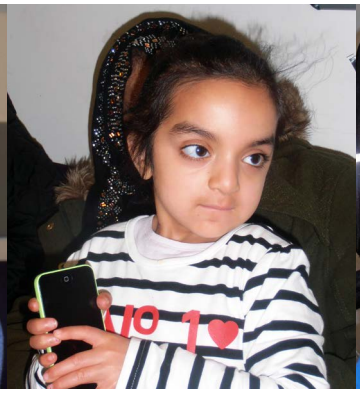
Morgan

Rubina

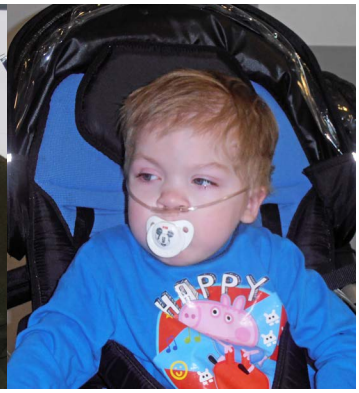
RMCH MPSI under 6 clinic - 29th January 2016

April

Avah

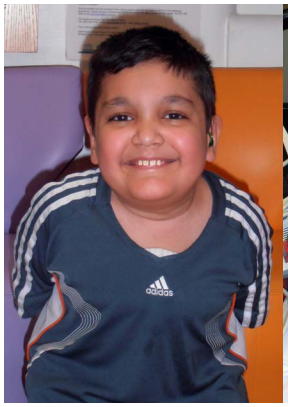


Hafsah



Mason

BCH MPS clinic - 5th February 2016



Asif

Mohammed

Roman

Sultan

Zena

The Care Act 2014 - An Overview

The Care Act 2014 came into effect in April 2015 and has been introduced in order to consolidate existing good practice guidance and legislation into a comprehensible document that directs and informs Adult Social Care in England; the legislation sets out clear objectives, rules and guidance about:

- Assessment
- Eligibility
- What people should be able to get
- Rights of individuals and their carers
- What councils will have to do

The underlying principles of the Act stem from concepts of well-being, integration, prevention and delay of the need for care services and for people to be in control of their care.

Well-Being

"Well-being" is defined within the act as being in relation to:

(a) Personal dignity (including treatment of the individual with respect);

(b) Physical and mental health and emotional well-being;

(c) Protection from abuse and neglect;

(d) Control by the individual over day-to-day life (including over care and support, or support, provided to the individual and the way in which it is provided);

(e) Participation in work, education, training or recreation;

(f) Social and economic well-being;

(g) Domestic, family and personal relationships;

(h) Suitability of living accommodation;

(i) The individual's contribution to society

The physical, emotional and mental well-being of both the **individual and their carer** will be considered.

Assessment

The Local Authority has a duty to assess where there is an apparent need for care and support for example, physical disability, learning disability, learning disability, frailty, mental health problems.

Assessments can now take varying forms and must be undertaken by appropriately qualified/ trained individual. Self-assessment can also be used; however Local authorities have a duty to over-see this to ensure that it is proportionate.

The information gathered during the assessment process will be used to establish care and support needs for individuals and their carers. These needs may include:

- Help to get out of bed, get dressed or washed
- Help with eating or cooking meals
- Help with seeing friends and family
- Help with caring for others

Care and support comes from lots of different people; family, friends, people in the community. The Local Authority will consider all your support options available to meet need not just traditional paid for services.

A financial assessment will be undertaken in order to determine any contribution toward care and support costs.

Eligibility

The Act has introduced an updated national eligibility criteria, however this criteria is still open to local interpretation. The Local Authorities are required to consider whether the person's needs:

- Arise from or are related to a person's physical or mental impairment or illness.
- Make them unable to achieve 2 or more specified outcomes.

- And if, as a result of the above, there will be a significant impact on the person's well-being.

The identified outcomes are:

- managing and maintaining nutrition, such as being able to prepare and eat food and drink
- maintaining personal hygiene, such as being able to wash themselves and their clothes
- managing toilet needs
- being able to dress appropriately, for example during cold weather
- being able to move around the home safely, including accessing the home from outside
- keeping the home sufficiently clean and safe
- being able to develop and maintain family or other personal relationships, in order to avoid loneliness or isolation
- accessing and engaging in work, training, education or volunteering, including physical access
- being able to safely use necessary facilities or services in the local community including public transport and recreational facilities or services
- carrying out any caring responsibilities, such as for a child

Support Services

The Local Authority has a duty to develop services appropriate to the needs of those identified as having support needs within their area. They are encouraged to work collaboratively and creatively with partner agencies and stakeholders to determine appropriate services.

The local authority must provide interim support in the event of service failure.

Personal budgets are a wary of planning and organising support provision. Individuals are offered a budget that is deemed to meet the identified needs and then receive this via a direct payment.

Transition

The Act places an emphasis on the need for seamless transition to adult services and states that;

"Where it appears to a local authority that a child is likely to have needs for care and support after becoming 18, the authority must, if it is satisfied that it would be of significant benefit to the child to do so and if the consent condition is met, assess—

(a) Whether the child has needs for care and support and, if so, what those needs are, and

(b) Whether the child is likely to have needs for care and support after becoming 18 and, if so, what those needs are likely to be.

The assessment must involve consideration of any current identified care and support needs and the likely impact of these once the child becomes 18 years old, the wishes of the individual in terms of achievement and outcomes and what care and support services could be made available to contribute to the achievement of those desired outcomes.

- The assessment must include parents and carers and any person that the child/ parents/ carers request e.g. The MPS Society.
- The assessment must take a holistic over-view of the child's and carer's situation.
- The assessment must indicate any support and care that the child is likely to need after becoming 18 and identify if these are likely to meet the eligibility criteria.
- Advice should be made available signposting to necessary support with an aim of delaying or reducing the need for support services.

Information, Advice and Advocacy

The Local Authorities have a duty to provide good information and advice about the help and support available in their area; they must also inform the public about assessment and eligibility, charging for services and financial advice, sign-posting to services.

The Act identifies the importance of independent advocacy and places a duty on the Local Authority to provide access to such services if a person is unlikely to be able to participate in needs assessment and support planning.

Safeguarding

The Act introduces Adult Safeguarding Boards and stipulates that Local Authorities have a duty to establish effective process for addressing safeguarding for vulnerable adults. There is an emphasis on collaborative working with partner agencies. The Local Authority must;

- **lead a multi-agency local adult safeguarding system** that seeks to prevent abuse and neglect and stop it quickly when it happens
- **make enquiries, or request others to make them**, when they think an adult with care and support needs may be at risk of abuse or neglect and they need to find out what action may be needed
- **establish Safeguarding Adults Boards**, including the local authority, NHS and police, which will develop, share and implement a joint safeguarding strategy
- **carry out Safeguarding Adults Reviews** when someone with care and support needs dies as a result of neglect or abuse and there is a concern that the local authority or its partners could have done more to protect them
- **arrange for an independent advocate** to represent and support a person who is the subject of a safeguarding enquiry or review, if required.

Financial assessment and paying for services

Within the assessments undertaken by the Local Authority there will a financial assessment. The Care Act is due to implement changes to this by way of a cap on the total charges an individual is liable to pay for their care however this has been postponed for 4 years. Information regarding the financial thresholds should be available through your local authority.

The Care Act 2014 will inevitably bring change for Adult Social Care with a particular drive toward better information, equity, prevention and personalisation. This leaflet is intended as an overview, should you have any questions about the Act please contact the MPS Society Advocacy Team.

For more information visit: https://www.gov.uk/government/publications/care-act-2014-part-1-factsheets

Great Ormond Street
LSD Multidisciplinary Clinics

Specialist Physiotherapist Michelle Wood and Clinical Nurse Specialist Sindi Mnkandla gave us the lowdown on multidisciplinary clinics at GOSH: what happens and why they are important.

What was the idea behind your multidisciplinary clinics?

Our multidisciplinary clinics have been running for several years now and were set up with the vision of providing an opportunity for multi-speciality professionals to be in the same room, working together with the family and allowing open communication and discussion of treatment plans.

One of the big driving ideas behind these clinics was to reduce the number of hospital appointments for our families and allow them to see several relevant professionals at the same time.

What happens at a multidisciplinary clinic?

The families will tend to arrive in the morning and have any investigations that are due – this may be blood tests, echo, lung function tests, x-rays etc - and they are seen in the clinic setting in the afternoon where they will have a consultation with various specialists, both doctors and allied health professionals.

A rough itinerary is provided and the families rotate round various professionals. One room houses the individual speciality consultants who see the patient together to promote maximum communication between the teams: the physiotherapist will assess such things as range of movement, muscle power and functional abilities; the speech and language therapist will assess swallow and any feeding issues; the psychologist will monitor development, behaviour and emotional well-being, as well as helping the whole family cope with the underlying disorder.

> *"We have worked hard to make these clinics as productive as possible. They are truly multidisciplinary with patients having the opportunity to see several specialists in one afternoon."*

The clinical nurse specialists also have a multitude of jobs. They will have already organised all the tests that need to take place, the clinic set up and itinerary, and then will make time to chat with the families and discuss any difficulties they may be experiencing. Afterwards the professionals meet together and there is opportunity for discussion and decision making to ensure all the needs of the patient and family are dealt with.

All LSD patients who have attended clinics (both the MDT and standard clinics) are also discussed by the wider team at our weekly MDT meeting. Here there is also valuable input from the laboratory team who help with diagnostics and biomarker monitoring, a social work team who will help with any specific difficulties the families are experiencing, and the pharmacy team who will discuss prescriptions and any changes to ERT dose etc.

How many clinics do you hold and how frequent are they?

We currently run five well established speciality MDT clinics:

- Pompe
- Niemann Pick C
- MPS III (Sanfilippo)
- MPS IVA (Morquio)
- Post Bone Marrow Transplant (BMT) (MPS I (Hurler) and other LSD)

We have recently held our first Fabry clinic for children with pain and a Gaucher Type III MDT clinic is planned for April 2016.

We hold 2 clinics a month on a Tuesday afternoon, 4 times a year for each specialty. We have 4 patients per clinic and the plan is for each family to attend a minimum of one MDT clinic a year. On average they will spend 3 hours in clinic.

What specialists are available at your multidisciplinary clinics?

In each clinic there are different specialties dictated by the needs of the patient:

Pompe: attended by metabolic consultant, neurologist, respiratory consultant, physiotherapist, speech and language therapy, clinical nurse specialist and Psychologist.

Niemann Pick C: attended by metabolic consultant, neurologist, respiratory consultant, physiotherapist, speech and language therapy, clinical nurse specialist and psychologist.

Post- BMT: attended by metabolic consultant, BMT consultant, orthopaedic consultant, neurosurgeon, cardiologist, physiotherapist, speech and language therapy, clinical nurse specialist and psychologist.

MPS III: attended by metabolic and psychiatric consultants, physiotherapist, speech and language therapy, clinical nurse specialist and psychologist.

MPS IVA: attended by metabolic consultant, respiratory consultant, neurosurgeon, physiotherapist, clinical nurse specialist and psychologist.

Fabry: attended by metabolic consultant, pain team, clinical nurse specialist and psychologist.

What sort of feedback have you had from families?

The MDT clinics can be quite a challenge as it is a very busy day for the families. There are a number of departments to visit and there is often a limitation of room capacity and space. It can be rather intense for them!

However, feedback from the families has been very positive:

- They like the fact all professionals are in one room discussing what is best for their child.
- Procedures can be prioritised and timed appropriately.
- It reduces the number of visits for different appointments.
- They value the opportunity to meet other families and the mutual support they offer.

Some of the comments families have reported to us:

"Came away well informed."

"Everything regarding our son's disease is now up to date and we know how things stand until next time."

"...got to see all of my child's doctors ...I felt reassured. It was brilliant."

"...nice to be able to see most of the doctors and other healthcare professionals in one appointment rather than having to make multiple trips to see various individuals which is both costly and takes a lot of time out of schooling/work."

We must acknowledge, however, that MDT clinics do not suit everyone - families are asked if they prefer the MDT clinics or individual appointments before booking in.

We are very grateful to the wonderful support from the MPS Society advocacy service that attend the clinics and greet the families in the waiting room. They provide invaluable support and advice to our families.

GOSH LSD MDT TEAM

Consultants : Dr Spyros Batzios, Dr Anupam Chakrapani, Dr Maureen Cleary, Dr James Davison, Dr Emma Footitt, Dr Stephanie Grunewald, Professor Paul Gissen.
Clinical Nurse Specialists: Ingry Camero, Sindi Mnkandla,
Psychologist: Dr Angela Simcox
Physiotherapist: Michelle Wood
Speech & Language Therapist: Gyani Swift
Laboratory: Katie Harvey
Pharmacist: Chin Gan
Social Worker: Elleni Ross

Michelle Wood, Specialist Physiotherapist & Sindi Mnkandla, Clinical Nurse Specialist.

(photos left to right: weekly LSD MDT meeting; the GOSH team meeting; Sindi and Ingry the Clinical Nurse specialists)

Remembrance

Hayleigh's Story

On the 22nd April 1998 our beautiful daughter, Hayleigh Louise Reynolds was born. A shining light was brought into our life.

At first everything seemed the way it should with a new baby, but as months passed Hayleigh started being quite sick and recurrent congestion together with glue ear followed. At first the doctor thought she had allergies, but felt she was too young to be tested. At 2 years she had her first operation for grommets, tonsils and adenoids. Following this surgery the anaesthetist commented on the stiffness of her neck and difficult intubation. It was put in her notes and she was passed on to the child development centre.

As Hayleigh passed all the development checks we noticed unusual differences between her and her older brother, but was told that no two kids develop the same way. During nursery Hayleigh's hearing became more of a problem and was sent to the education audiologist. This proved to be a godsend as the audiologist involved recognised Hayleigh's' facial features and advised us to push for genetic testing.

When Hayleigh was five years old we eventually got the diagnosis of Hurler Scheie disease and were introduced to her consultant Dr Peter Robinson. Immediately he applied for ERT but was unsuccessful. We were put in touch with the MPS Society and through all the hard work and dedication of Christine and colleagues, together with lawyers Anderson & Strathearn, we eventually took Greater Glasgow Health Board to court and won. It only took 3 years. It made it easier for future families to be granted treatment. Hayleigh enjoyed the attention of being on television.

Hayleigh started her ERT treatment and things seemed to settle down for a few years. Operations came and went and Hayleigh never complained even when new medicines were introduced and her pain was so severe she needed morphine. As she became a teenager her mobility decreased and eventually had to rely on a wheelchair full-time, but it didn't faze her at all she just carried on going to concerts, shopping and all the usual things girls her age did.

In July 2014 Hayleigh became very poorly and spent a week in intensive care. We were told that Hayleigh's heart and lungs were deteriorating but no operation would be offered as the side effects were too great and she wasn't strong enough to survive. A second opinion was sought from Manchester Children's Hospital but the answer was typically the same. Take Hayleigh home and enjoy spending time with her.

Again Hayleigh surprised everybody involved and rallied round. Weekly infusions at Yorkhill continued. In February 2015 Hayleigh woke up and complained that she couldn't see. Panic set in and it was confirmed that the corneal clouding had taken away all of her sight in the left eye and most of the right. Hayleigh took all this in her stride, the only

thing she fussed about was that she couldn't see to do her art and when was she going back to school.

April came and again Hayleigh became increasingly unwell together with temporarily losing her memory. This time we were told it was a matter of time. The hard decision to make was to tell Hayleigh how poorly she was, from talking to people we decided against it. Yorkhill hospital put us in touch with Robin House Children's' Hospice in Balloch where we went for a visit. This was a daunting thought but entering through the front door we felt a huge hug encircle us and realised that we needed this kind of support. The hospice was a place for living and one of the best things that Hayleigh loved was the gardens and the fountain outside her bedroom. The next 6 months were hard going, but as before Hayleigh would not let this affect her all the time and continued getting on and being a teenager.

On the 21st November Hayleigh was taken to hospital because of pins and needles in her leg together with exhaustion and breathing problems, she needed constant oxygen together with her bipap ventilator. It became apparent nothing was helping and decided to transfer her to Robin House. The 3 days she was at Yorkhill every nurse and doctor that worked with Hayleigh visited to say goodbye.

The hardest part was to sit down with Hayleigh and explain that she was going to die.

Hayleigh spent the next few days enjoying time with the clown-doctors, friends and her family but sadly passed away on Sunday 29th November 2015 aged 17 years.

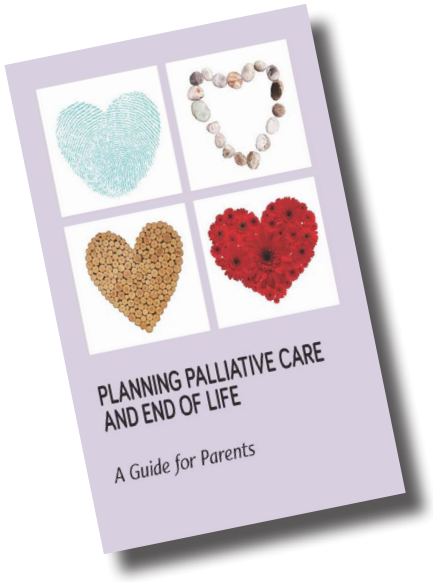
We stayed at Robin House for 5 days until her funeral, I know it doesn't sound right but it was a lovely time, we got to spend quality time with her to say goodbye.

> *"Hayleigh inspired everyone she met and showed people that no matter how bad you feel, always smile"*

We have had lots of lovely messages about how Hayleigh inspired everyone she met and showed people that no matter how bad you feel, always smile.

Our light has gone out but we still have the sparkle that she left behind.

Robert & Heather Reynolds



Planning Palliative Care and End of Life Booklet

If you are parent to a child needing palliative care, please get touch to request a copy of our booklet, *Planning Palliative Care and End of Life - A Guide for Parents*.

This booklet has been written to try to provide you with information and help with both practical and emotional issues surrounding end of life care, loss and bereavement. It has been written primarily for families facing the death of a child, young or old, but also for partners facing the loss of a loved one.

Our Advocacy Team are also on hand to provide you with support with palliative care and in the event of a bereavement.

Please call 0345 389 9901 or email info@mpssociety.org.uk to request a copy of *Planning Palliative Care and End of Life*

Bereavements:

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

Elizabeth Kirkpatrick who suffered from Morquio disease and passed away on 8th February 2016 aged 45 years.

Lorraine Rock who suffered from Morquio disease and passed away on 25th January 2016 aged 41 years.

LADY SHAUNA GOSLING

1937 - 2016

It is with great sadness that we learnt of the death at the age of 79 of a very good friend and much appreciated supporter of the MPS Society, Lady Shauna Elizabeth Gosling. Shauna, as we fondly knew her, died whilst on holiday in Langkawi in Malaysia in January. She was a devoted mother to Mark, Adam and David and beloved Grandmother to Luke, Alexander, Oliver, Sophie, Poppy, Leo and Zoe and will be hugely missed by all those who knew and loved her.

Christine Lavery

Oh, To Be A Dormouse

Starkness of the trees without leaves
gives us warning that winter approaches.
No more kicking the burnished copper leaves into the air
No more picking ripe blackberries from the bushes
to mix with round bitter apples.
Gone are the plums, and all summer fruits
Vegetables change too from peas and beans to parsnips and sprouts!
Fruit is down to apples and pears unless looking to afar for fruits
that can never be as succulent as ours in summer
Rain and wind is gusting outside
It is cold and grey and real english winter.
Oh to be a dormouse, to snuggle up warm in a cosy place,
only surfacing when spring arrives with its cheerful scenes
flowers appearing out of the ground, the sun may be weak
but it is there, breaking through the hovering clouds
Yes, dark winter, please pass quickly
And let us emerge with spring.

By Lady Shauna Gosling

Your Stories

Sally Mitcham recently submitted a poem to 'Hour of Writes' about her son Danny who suffers from MPSII Hunter disease. Sally's poem was selected as a featured entry, and we have reprinted it below.

If you have written a poem or short story about your experiences with MPS, Fabry or a related disease, we would love to see it! Email us at magazine@mpssociety.org.uk.

Youth Of Today

I see them
Loping along in packs
Long legs eating up the asphalt.

I see flocks of them
Swooping across the road on two wheels
Heedless of oncoming traffic.

I scowl and want to shout Take more care!
Life is more precious than they realise.

I see them
Huddling on a freezing corner
Dragging on a cigarette
Hurling insults and obscenities at each other.

I see them
Prowling their territory
Preening and edging around the other sex.

I warm to these signs of frailty
The first fumbling steps in the courtship dance.

I see them
Moving aside for our buggy
Glancing at my son
as he gabbles an incomprehensible greeting.

Do they see his otherness
A simple mistake in tangles of DNA that has diverted his future
Waste products building up in this body I love so much
Grinding his brain to a halt?

I wish I could see further
See him strutting with the other lads
Being teased by a girl.

I wish I could see a woman like me
watching him with disapproval and tutting
Muttering under her breath, 'Youth of today...!'

Sally Mitcham
(first published on Hour of Writes at hourofwrites.com)

Matthew's Amazing 12 Months

Matthew is coming up to his 18th birthday, he is currently studying his btech sport level 3 at Aston Academy in Rotherham, where he is on target to have the course completed by mid April!

When not at school Matthew's great passion is cricket!!!

Matthew started playing cricket at our local club, Treeton CC after being introduced to the game through playing on our neighbour's back garden with his grandson. Treeton CC is a special place for Matthew and he loves being there, either playing, watching or socialising!

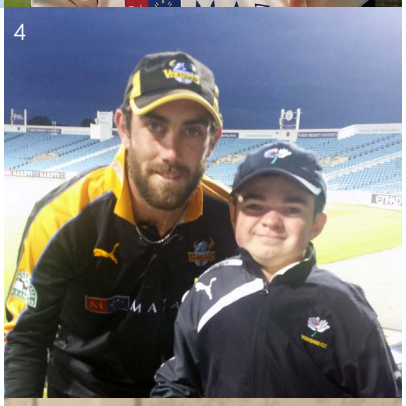
He quickly learned the art of wicket keeping to become the team stumper and is also a talented batsmen

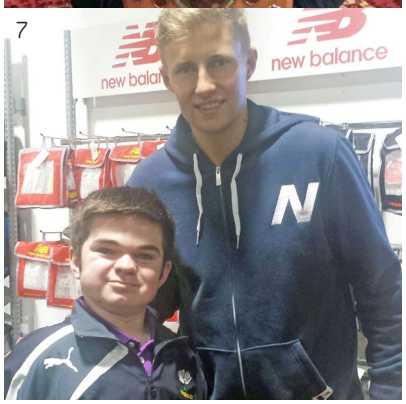
His batting and wicket keeping skills progressed and Matthew went on to also represent Yorkshire CC disability section, playing games all over the country.

Cricket has brought some fantastic opportunities Matthew's way over the last 12 months. Matthew is now thinking about doing a bit of cricket coaching as his next step in his cricket passion!

We are soooo immensely proud of Matthew for all he is achieving and just for his general attitude to life; all who meet him take to him. Who knows what lies ahead in Matthew's sporting life...watch this space!!!

Russell Ingram
Matthew's dad





Photos: 1. meeting one of his favourite players, England's James Taylor; 2. Matthew sitting proudly with his Leanne Knight awards he had presented to him at Aston Academy for his outstanding attitude, effort and achievement at school and away from school; 3. Matthew wearing the white rose of Yorkshire just before taking to the pitch for a game; 4. Meeting Yorkshire and Australia cricket superstar Glenn Maxwell; 5. Matthew being presented the cricket OSCA as Disability Achiever of the Year; 6. holding the Treeton CC John Foster award he won at Treeton CC presentation night last November for his ongoing achievements in cricket; 7. Matthew meeting his hero, cricket superstar Joe Root.

Welsh Information Day 2016

Being only weeks new to the MPS Society, I was excited to attend my first information day on MPS diseases and be part of the running of the day. I was not disappointed and thoroughly enjoyed meeting the various professionals, parents, young people with the disease and a trustee. I found that the information day really helped me to start to piece together some of the information I had been given about the varying MPS diseases, how these are inherited, and what treatments and support could be offered.

It was useful to hear from a senior physiotherapist and a specialist nurse about the treatment that was available to help realign the spine and legs, and how to care for gastrostomy sites. A few Consultant Metabolic Paediatricians also shared some information about pain management for Fabry sufferers, and information about current and future clinical trials. This information was useful as it helped me to start to gain some insight into the support that is available to sufferers and families.

The information given by a Consultant Paediatrician Clinical Psychologist in Neuropsychology was extremely well presented and I could see how families could identify with what was said and may have been able to build on their own self-help techniques to managing behaviour.

I found the families stories particularly interesting, and I hope to support my colleagues in the future with developing some more resources to support the siblings of the children and

young people and adults with the disease. Information from a Family Support Practitioner from Ty Hafan Hospice, and many other professionals and families really helped me to begin to grasp the journey that families with rare MPS and related diseases face.

As I continue to learn about and support those with MPS and related diseases, I hope to meet many more families and professionals at information days and conferences so that my learning is enriched, and I can offer a higher level of support.

Louise Cleary
Advocacy Support Officer

The MPS Society invites you to apply for a family trip to Lapland!

The trip will take place in December 2016 for a limited number of MPS families, consisting of up to 2x adults and 2x children (aged 2 - 14 years).

This trip of a lifetime includes return flights from Gatwick Airport to Kittila Airport in Finland, 3 nights hotel accommodation (including breakfast and evening meals), sleigh rides, tobogganing, sledging through the winter wonderland, a visit to Santa Claus' workshop and sorting office with the opportunity to give Santa your letters in person (subject to change at time of booking), and much more.

This trip promises to be a magical experience, remembered forever by all.
Look out for the application form in this issue of the magazine

RARE DISEASE DAY 2016

Many of you will have been aware that Monday 29th February 2016 was the ninth International Rare Disease Day. Each year we are one of the hundreds of patient organisations that get involved to raise awareness for this important day that is all about putting rare diseases, like MPS, Fabry and related diseases, in the spotlight.

Eurordis, the organisation behind Rare Disease Day, was pleased to announce that 2016 saw the day reach new heights, and on a global scale. Events were held in 85 countries and regions (all 28 EU countries!), with some newcomers to Rare Disease Day in the form of Andorra, Aruba, Indonesia, Libya, Mauritius, Moldova, Tanzania, Tunisia, Uganda and Zimbabwe, proving that year on year Rare Disease Day is getting bigger and better!

It was not just patient organisations that got involved - researchers, medical professionals, carers, healthcare employees, policy makers and institutions also added their voices to the mix, helping to bring rare diseases to the attention of governments and the general public.

While involvement was truly global, it was also digital, with many people and organisations taking to social media to help spread the word about Rare Disease Day. In fact the online campaign was so successful that #RareDiseaseDay trended on Twitter, with over 36,000 tweets going out with this hashtag and on Facebook 6 million people were reached on the day! The MPS Society were delighted to be able to take to our social media to help support this very worthy campaign, and our thanks to all those who liked and shared our Rare Disease Day posts!

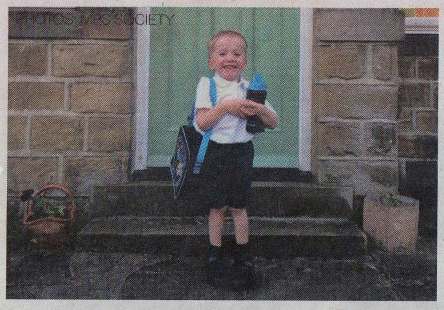
MPS Society in the Media for Rare Disease Day 2016

To celebrate Rare Disease Day, each year *The Independent* contains a supplement by Media Planet that is dedicated to rare diseases, featuring everything from rare disease medicine commissioning and research to patient stories.

For 2016 the MPS Society were fortunate enough to be given the opportunity to feature two adverts within the supplement, helping to raise awareness for MPS, Fabry and related diseases, along with the vital work of the MPS Society. In addition to this, Katy Brown, mother to Sam Brown and passionate campaigner for the funding of MPSIVA drug Vimizim, was asked to write an article about the journey taken by herself, the MPS Society and the other campaigners involved, and why Vimizim was worth fighting for.

Our thanks to BioMarin and Shire for supporting this initiative and helping us to educate more people about MPS, Fabry and related diseases.

PHOTOS: MPS SOCIETY



December 2015 marked a turning point for the Morquio community, with NICE's approval of the drug to treat the disease

We have kept Sam smiling

The power of a collective voice can make a real difference to people's lives

By Katy Brown, mother of an MPS Child

"My seven year old son Sam walked towards his classroom, on his back; a rucksack looking over-sized on his diminutive frame. Inside, not a selection of books but more surprisingly a pump and drip bag containing a drug we've been fighting for the NHS to fund for over a year, topping him up with a man-made replacement for an enzyme that his little body can't produce.

Sam was born with an ultra-rare condition called Morquio disease which is genetically inherited, and has just 105 sufferers in the UK. Morquio causes progressive physical disabilities including hearing and sight problems, and ultimately respiratory and cardiac complications causing early death. There is no cure.

Sam doesn't grow properly. His three year old brother is about to overtake him. Height is not a popular topic of conversation in our house and despite Sam's stoic view on life his size bothers him.

In 2012 Sam was given the opportunity to take part in a clinical trial for a novel treatment for Morquio. Four years on and the treatment has changed his life. Sam is more physically capable than ever and continues to grow slowly providing clear evidence that the drug is successfully slowing the progression of the disease and improving quality of life.

I never imagined that the hardest part of our journey, including that dreadful day of diagnosis, would be the battle we have had over the past 12 months to get the drug funded by the NHS. It arrived for approval at exactly the wrong time. The reorganisation of the NHS meant that the previous clear pathway for drug approval for ultra-rare diseases had been dissolved with no tangible replacement agreed. Accountability between NHS England, NICE and the Department of Health was grey which led to delays, broken promises, uncertainty and the economic pressures affecting the NHS were, and still are, front page news.

One of the founding principles of the NHS is universal healthcare. Drugs for rare diseases are fundamentally expensive because there are so few patients to spread the very significant development costs over, which is bad luck for sufferers. If the NHS chooses to say no to drugs simply on the basis of cost it may as well tell all rare disease sufferers on diagnosis that sadly they are too rare and expensive to treat. That certainly is not the NHS as I recognise it.

As a mum, having someone place a value on my son's life is a terrifying concept. But sadly that is exactly what we have been subjected to over the past 12 months of campaigning. We and other families have been through the ringer, but we have never ever given up. Failure was never an option.

Finally on the 16 December 2015, the news we had been eagerly awaiting was finally announced. The drug was approved by NICE for five years subject to criteria that patients need to achieve. Cautiously the Morquio community breathed a sigh of relief.

"Morquio disease is genetically inherited, and has just 105 sufferers in the UK"

As I reflect on 2015, I feel proud of what we have achieved. Last summer NICE were recommending a 'No' and the MPS Society and a small number of incredibly passionate, determined people, managed to turn the supertanker around. It's not a perfect solution, we still have to wait to see if Sam and others will be able to pass the narrow criteria set in 12 months' time to continue treatment. However, I genuinely believe that we have achieved the very best that we possibly could. Sam and other Morquio sufferers now have the opportunity of a far better quality and length of life. Quite frankly, there is no more worthy a fight than that. ■

Read more on healthawareness.co.uk

Katy Brown's article on the Vimizim campaign and why it was important

One of the MPS Society adverts that appeared in Media Planet's Rare Disease supplement, distributed by The Independent

Research & Treatment

Fabry Disease Research and Treatment

Amicus Therapeutics oral therapy, Migalastat, is being appraised through the NICE highly specialised technologies process. The MPS Society is leading the patient submission for the use of Migalastat in Fabry disease.

FDA requires more data on Migalastat

FDA fast-tracks development of Genzyme oral drug, GZ/SAR402671a new investigational oral substrate reduction therapy for the treatment of Fabry disease.

Japanese ERT to use cloud tech in Fabry research – Medidata, the leading global provider of cloud-based solutions for clinical research in life sciences has announced that its cloud-based technology platform has been adopted by JCR Pharmaceuticals. The pioneer in biotherapeutics is leveraging the Medidata Clinical Cloud to support research on a therapy for the treatment of Fabry disease, bringing greater speed and operational efficiencies to the organisation's development programme in Japan.

Protalix announces positive clinical trial results – Protalix BioTherapeutics has announced interim data from the company's phase I/II clinical trial of 1mg/kg of PRX-102 for a treatment of Fabry disease. PRX-102 is a recombinant plant cell expressed, chemically modified version of the human alpha-Galactosidase-A enzyme. The phase I/II clinical trial of PRX-102 for the treatment of Fabry disease is an open-label, dose-ranging study treating up to 18 naïve male and female adult patients.

Trial to start for moss-made ERT for Fabry disease – Greenovation, a German Biotech company, has announced a phase I clinical trial application for its drug candidate moss-alpha-galactosidase (moss-agal).

NICE's Quality Standard

NICE has released a quality standard focusing on caring for children, young people and adults with a learning disability and behaviour that challenges.

This quality standard highlights that behaviour that challenges is often considered a result of a persons' interaction between environmental and person factors. The expectation of the standard is that it will contribute to the following outcomes of: safeguarding, personal dignity, experience of care, premature mortality and patient safety.

The standard provides eight quality statements all focusing on the care people with learning disabilities receive. Families and carers are recognised as having an important role in supporting those with a learning disability and behaviour that challenges and where it is appropriate the health and social care practitioner should make sure they are involved in the decision making process when caring for, investigating and treating the patient.

The standard recognises that parents and carers often have insufficient support from professionals who do not help early enough and don't have the necessary expertise and focuses on how to ensure that assessments of children and adults will lead to them receiving personalised care planning. The standard also makes a range of statements which are aimed at ensuring that the approaches used by staff involved with children and adults with learning disabilities support them, while following the least restrictive practice they can.

www.nice.org.uk/guidance/qs101

BUPA Home Healthcare Change of Ownership

The owner of Bupa Home Healthcare is changing to Celesio later this year. Bupa Home Healthcare have advised the MPS Society that Celesio has a range of supporting healthcare services delivered in partnership with the NHS and pharmaceutical companies. We are assured that Celesio shares Bupa's commitment to patients and is dedicated to delivering a high level service.

I understand from Bupa that ownership changing will not affect the arrangements that are already in place for patients care. Patients or their parent / guardian will be informed by letter in due course.

If you have any questions please do in the first place speak to your usual Bupa home healthcare contact. If you still have questions please speak to the LSD team at your LSD expert centre or contact the MPS Society.

REGENXBIO Inc.

MPSI

REGENXBIO Inc. is a leading biotechnology company focused on the development, commercialisation and licensing of recombinant adeno-associated virus (AAV) gene therapy.

The company's mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercialising in vivo gene therapy products based on their NAV Technology Platform. RGX-111 is REGENXBIO Inc.'s product candidate for the treatment of Mucopolysaccharidosis Type I (MPS I).

Individuals with MPS I have a deficiency of alpha-l-iduronidase (IDUA), an enzyme found throughout the body, including in cells in the central nervous system (CNS) that is responsible for the breakdown of polysaccharides. RGX-111 uses the AAV9 vector to deliver the IDUA gene to the CNS creating the potential that normal IDUA enzyme can be produced. Delivery of the gene encoding the enzyme could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long term cross-correction of cells throughout the CNS. This strategy could also provide rapid IDUA delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in MPS I patients.

The U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation and Rare Pediatric Disease Designation to RGX-111 for the treatment of MPS I. Initiation of a Phase I/II clinical trial is planned for mid-2016.

For more information, visit http://www.regenxbio.com/.

MPSII

RGX-121 is REGENXBIO Inc.'s product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), also known as Hunter syndrome.

Individuals with MPS II have a deficiency in the lysosomal enzyme iduronate 2-sulfatase (IDS), an enzyme found throughout the body, including in cells in the central nervous system (CNS) that is responsible for the breakdown of polysaccharides. RGX-121 uses the AAV9 vector to deliver the human IDS gene to the CNS creating the potential that normal IDS enzyme can be produced. Delivery of the gene encoding the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDS beyond the blood-brain barrier, allowing for long term cross-correction of cells throughout the CNS. This strategy could also provide rapid IDS delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in Hunter syndrome patients.

The U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to RGX-121 for the treatment of MPS II.

For more information, visit http://www.regenxbio.com/.

New Drug to Treat Morquio Syndrome to be Made Available in Wales

16 March 2016, Welsh Government

A new drug to treat Morquio Syndrome, a rare life-limiting inherited disease, will be available on the Welsh NHS, Health and Social Services Minister Mark Drakeford announced today (Wednesday 16th March 2016).

The Minister has approved a recommendation from the All Wales Medicines Strategy Group (AWMSG) that Vimizim® (elosulfase alfa) should be available in Wales.

Morquio Syndrome is a very rare inherited disease. The signs and symptoms of the condition are usually not obvious at birth but start to appear in the early years of a child's life. These include oddly-shaped bones, knock knees, spine curvature and irregular chest growth.

As a child with Morquio Syndrome gets older, more serious symptoms start to become apparent and more significant and multi-systemic clinical impairments develop. This can cause pain, fatigue, diminished functional capacity, decreased endurance and impaired quality of life. People with Morquio Syndrome have a life expectancy of around 30 years.

Before Vimizim® was introduced, the only treatment option was palliative or supportive care, which did not treat the underlying cause of the disease. Vimizim® is the first treatment which has the potential to alter the course of the disease.

The cost of treating all eligible patients in Wales is estimated to be £880,000 in the first year.

Professor Drakeford said:
"I'm pleased to be able to ratify the All Wales Medicines Strategy Group's recommendation that Vimizim®, which is the first treatment which has the potential to alter the course of Morquio Syndrome, should be available in Wales.

"Clinical experts suggest the therapy is expected to slow disease progression, reduce the need for surgery and improve quality of life.

"I'm sure the approval of Vimizim® will be welcome news to people and families living with Morquio Syndrome."

All medicines approved by the National Institute for Health and Care Excellence or AWMSG are made available routinely in NHS Wales where clinically indicated.

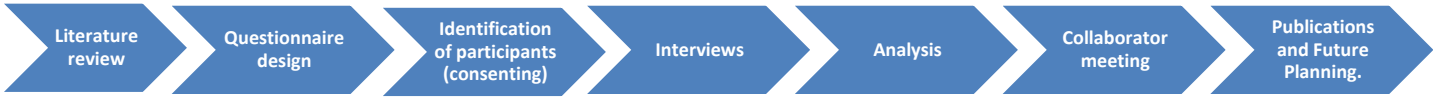
The experiences and information requirements of women with an MPS or related condition during conception, pregnancy, birth and the postnatal period.

Alison Wilson[1,2] ; Sophie Thomas [1]; Debbie Cavell [1]; Rebecca Brandon [1]; Dr Elaine Murphy [5]; Dr Fiona Stewart [2]; Dr Vikram Talaulikar [3]; Dr Gisella Wilcox [4]; Joanna Wilson-Smale

[1]Society of Mucopolysaccharide Diseases, Amersham, UK, [2] Northern Ireland Regional Genetics Service, Belfast Health and Social Care Trust, Belfast, UK, [3]Reproductive Medicine Unit, University College London Hospital, London, UK[4] Salford Royal Foundation Trust, Manchester, UK[5]Charles Dent Metabolic Unit, National Hospital for Neurology and Neurosurgery, London, UK

Study outline

The aim of this study was to, for the first time, interview women with MPS and related conditions who have had successful pregnancies in order to gain a meaningful insight into their needs and experiences during this time. It was also the aim of our group to take steps toward developing guidelines for the management of these complex pregnancies and to develop information resources for women with an MPS or related condition of childbearing age. Figure 1 outlines the study protocol. The study recruited 6 participants (3 conditions represented: MPS I (HS), MPS I (S) and MPS IV; both treated (Enzyme Replacement Therapy) and non-treated. Interviews comprised of 5 core sections: General, Family History, Preconception and Planning, Pregnancy and Birth, Postnatal Care.



(Figure 1)

Planning and Conception

Participants' experiences of preconception counselling were mixed; with some receiving no preconception counselling at all while others spent time in detailed conversation with their metabolic consultant.

Although all participants demonstrated a good knowledge of the genetic inheritance of their condition only one participant received formal genetic counselling and support when she was planning her pregnancy – this participant described a positive experience and felt 'empowered'.

No participants in the study experienced any fertility problems.

Information Requirements

The recommendation of this group is to incorporate preconception and genetic counselling into routine transitional care to ensure that all individuals of childbearing age are fully aware of their options.

The MPS Society are developing patient information resources to support family planning and signpost women of childbearing age to appropriate services.

Participant quote:

'....my parents were told when I was little that I wouldn't live long enough to have children. So I didn't even think about it until I was married. Then I met my husband and panicked about our future. It would have been helpful to have had the conversation earlier.'

Postnatal Care

Participants described varying experiences of postnatal care and recovery. Some new mothers were able to return to normal activities soon after birth while others required significant/intensive care and support to look after themselves and their new baby. Pain, fatigue and reduced mobility (including the requirement for mobility aids) were the most limiting factors in the postnatal period. Although participants described excellent care and support by midwifery and nursing staff there was a clear gap in MPS-specific knowledge. Again there were variations in advice relating to frequency of review appointments, the use of ERT (particularly while breastfeeding) and the availability of social support.

Information Requirements

The recommendation of this group is that the medical community develop clear guidelines for the use of ERT in the postnatal/breastfeeding period. It is also essential that clear guidance is provided in relation to the frequency of review in the postnatal period. Specific training for midwives and community nursing staff should be developed.

Participant quote:

'After the birth I was fairly immobile. I needed to use a wheelchair until my back improved. I also really struggled with big decisions, like whether or not to breastfeed. No-one could give me any advice about breastfeeding while on ERT so the decision was in my hands – that's a lot of responsibility for a new mum! What if I got it wrong?'

Society for Mucopolysaccharide Diseases
www.mpssociety.org.uk

Pregnancy and Birth

Participants described having typical early pregnancy problems (including sickness, back pain and fatigue). The key difference with these women was the level of anxiety about *'what might happen'* in addition to uncertainty surrounding how their pregnancy should be managed. Participants described differing levels of follow-up, approaches to pain management, advice about medications (specifically the use of Enzyme Replacement Therapy) and advice about birth. These differing experiences left mothers-to-be feeling confused and often frustrated with the system designed to support them.

Exacerbation of MPS issues such as orthopaedic pain and respiratory stress were common problems for participants.

Lack of interaction between specialties and lack of understanding of MPS was a key concern.

Information Requirements

The recommendation of this group is that the medical community develop peer reviewed guidelines for the management of these complex pregnancies.

We would advocate for the introduction of a pre-pregnancy preparation clinic (a joint Metabolic/Obstetrics Clinic) for women with MPS. This clinic should include a medication review, pain management discussion, pregnancy risks discussion and preliminary birth planning. At this stage women should be referred for baseline investigations (cardiac, respiratory and spinal) and should receive genetic counselling. This should ensure that women feel informed and empowered at the start of their pregnancy and that their on-going care is mapped out.

Participant quote:

'It was exciting to be pregnant! But it was scary not knowing what to expect. It was even scarier that my doctors didn't know either.'

Bringing baby home

All participants recounted how MPS impacted on being a new parent; with some finding the practicalities more challenging than they had anticipated. Fatigue, pain and physical restrictions made day-to-day tasks more difficult to manage. Specific difficulties discussed by participants related to manual handling, feeding, bathing, changing, managing equipment and navigating their home with a new baby in their arms. Although some new mothers had excellent support from family, others were somewhat isolated during this period. Only one participant was offered social support from statutory agencies.

Information requirements

The recommendation of this group is that patient resources are developed in conjunction with experienced MPS parents to provide tailored practical advice. The MPS Society are developing these resources for publication. It is also recommended that information for healthcare professionals is developed to support a more in depth understanding of the difficulties these new parents face. It is hoped that by improving awareness new parent will be offered more tailored support packages.

Participant quote:

'It was a bit of a shock to be home with a baby. I found everyday things like getting her dressed harder than I expected. I couldn't manage to carry her upstairs; so our living room became a nursery! I had wonderful support from my family; but my community nurse wasn't really sure how to help'

Pregnancy experience poster as displayed by the MPS Society at the 12th annual WORLD Symposium (an interdisciplinary forum related to LSDs), held in San Diego, California in March 2016.

FABRY REPORTED OUTCOMES SURVEY

THE PATIENT'S EXPERIENCE OF FABRY DISEASE AND TREATMENT

357 Fabry patients on the MPS Society Registry were invited to participate in a telephone interview / written survey to provide the patient experience of Fabry disease and treatment 174 Fabry patients participated. (49% response)

The survey study was funded from MPS Society's own resources. The study was not supported in anyway by any pharmaceutical company. No patient was offered a financial incentive to participate.

Number of Fabry patient participants - **174**
Males - **77**
Females - **95**
Not known - **2**

The age of participants ranged from 3 years to 85 years (mean 41 years)

Participants cared for by an Expert Centre:

Adults (≥16 years) (N=154):

Addenbrookes Hospital, Cambridge - **13**
Addenbrookes/Royal Free - **1** (shared care)
City Hospital, Belfast - **14** (includes 2 x 16 year olds)
Belfast/Royal Free - **1** (shared care)
Birmingham University Hospital - **12**
University Hospital, Cardiff - **7**
Great Ormond Street Hospital - **2** (2 x 16 year olds)
National Hospital, London - **16**
Manchester Children's Hospital - **1** (17 years old)
Royal Free Hospital - **48**
Salford Foundation Hospital - **33** (one 16 year old)
Salford/Royal Free - **1** (shared care)
Not Known - **5**

Children (<16 years) (N=20)

Birmingham Children's Hospital - **5**
University Hospital, Cardiff - **1**
Great Ormond Street Hospital - **7**
Manchester Children's Hospital - **3**
Royal Free Hospital - **3**
Not Known - **1**

No. on ERT - **128**
Fabrazyme - **53**
Replagal - **75**
No Answer - **1**
No. Not on ERT - **41**
No. Not known - **3**
No. on Migalastat - **1**

No. affected on Fabrazyme at start of shortage due to stopping treatment or dose reduction in Fabrazyme - 54

- *My pain levels increased so much when I missed doses that I had to switch*
- *Increased pain in hands / feet. Increased fatigue, headaches and stomach cramps*
- *Increased fatigue (2)*
- *More fatigue, increased IBS, increased headaches*
- *Had 0.5 mg/kg for 6 months and felt very unwell, improved on 0.2 mg/kg of Replagal*
- *Suffered a second stroke. Consultant suggested was due to drug change*
- *Bad stomach aches, tired and fatigued*
- *Extreme fatigue, loss of strength, lower immune system, increased breathlessness*
- *Increase in GI symptoms*
- *Developed dry eye syndrome, more crises, itching, nausea, wheat intolerance*
- *Symptoms increased when my treatment went to four weekly and before I changed to Replagal*
- *Due to no treatment suffered TIAs and in Dec 2009 had a major stroke. Changed to Replagal and had major stroke in Sept 2010*
- *Pain increased and unable to work*
- *Increase in pain and GI symptoms*
- *Had TIA in Oct 2012*
- *Very tired and acroparathesia increased when not on Fabrazyme*
- *Heart condition worsened rapidly requiring bypass and valve replacement*
- *Dizzy spells, nausea, heavy body and arms*
- *Stopped sweating and felt unwell all the time*

No. switched from Fabrazyme to Replagal due to shortage - 44

- On Replagal I had anaphylactic shocks, increased fatigue and heart episodes
- Suffered reactions on Replagal so was put back on low dose Fabrazyme then full dose after shortage.
- Did not feel as well on Replagal
- Doctor advised I stopped Replagal as symptoms very bad so was without treatment for sometime
- GI symptoms improved. Raynaud's increased. LVH stopped shrinking on Replagal
- Had adverse reactions to Replagal. Could not start on Fabrazyme due to shortage but after reaction was changed to Fabrazyme

No. who changed back to Fabrazyme at end of shortage - 20

Are you diagnosed with LVH?	Yes - **73**	No - **66**	Not Known - **35**
Have you had any strokes?	Yes - **17**	No - **129**	Not Known - **28**
Have you had any TIAs?	Yes - **22**	No - **121**	Not Known - **31**
Are you on dialysis	Yes - **1**		
Are you waiting for a kidney transplant		Yes - **1**	
Have you had a kidney transplant		Yes - **3**	

Vimizim Managed Access Agreement – what's the latest?

In the Winter 2015 issue of the MPS magazine, we were delighted to share the news that the Vimizim Managed Access Agreement (MAA) had been given the go ahead by NHS England and although the decision on an individual basis was 'No' in Scotland, all four children have had access to Vimizim approved.

Then, just this week, we received some more good news – the Welsh Government has agreed to fund Vimizim! For those of you in Northern Ireland, please be assured that we are continuing to fight your corner, so watch this space.

What is the MAA all about?

Vimizim has shown some promising results in clinical trials, however, as yet, we don't have information on long-term treatment and its benefits. For this reason, NICE would not agree to fund Vimizim without having a set of conditions in place. This led to the development of the Managed Access Programme (MAP) which is a completely new way for individuals to get access to treatment whilst long-term data are gathered.

Who can sign up to the MAP?

Entry into the MAP is dependent on a number of criteria – not everyone is eligible.

Criteria which prevent access to the MAP

The MAP isn't available to individuals who:

- Are diagnosed with an additional progressive life-limiting condition where treatment would not provide long-term benefit , e.g. cancer or multiple sclerosis

  Or

- Have a lung capacity (FVC) of less than 0.3 litres and requires ventilator assistance

  Or

- Are unwilling to comply with the associated monitoring criteria

Criteria to access the MAP

Individuals:

- Must have a confirmed diagnosis of MPS IVA
- Must have a confirmed enzymatic test, elevated urinary keratan sulfate, and mutation analysis
- Will sign up to the Managed Access Patient Agreement

What happens on the MAP?

Expectations whilst on the MAP

On the MAP, an individual will be expected to:

- Have a full set of baseline data collected*
- Attend clinic three times for assessment within a 14 month period

**Before treatment starts*

In addition, individuals will be required to complete a series of questionnaires at regular intervals, to collect information on their quality of life (QoL). Questionnaires will be carried out by Alex and Jackie, the MAP Support Officers. The questionnaires last approximately 40 minutes and are usually conducted over the telephone, although clinic and home visits can be arranged in special circumstances. Interpreter services are also available.

Individuals need to meet a number of criteria to remain on treatment. If an individual has their treatment stopped, they will be monitored for progression and will continue to be assessed, as this will allow gathering of important information from individuals who are not on treatment.

Stopping treatment

Individuals will stop treatment if they:

- Are non-compliant with assessments
- Fail to meet four out of five clinical criteria
- Are unable to tolerate infusions due to infusion-related severe adverse events that cannot be resolved

How to enrol

If you feel that either you or someone you know would be eligible to join the MAP and are interested in enrolling, please contact Alex or Jackie.

Telephone: 0345 389 9901
or
Email: mps@mpssociety.org.uk with Managed Access Programme in the subject heading

Jacqueline Adam & Alex Morrison
Managed Access Programme Support Officers

Lysogene to Host First-Ever Research Symposium Dedicated to GM-1 Gangliosidosis

Partners with Cure GM-1 Foundation and the National Tay-Sachs and Allied Diseases Association

Paris—March 9, 2016—Lysogene, a leading clinical-stage biotechnology company developing gene therapy for rare disease, today announced its sponsorship of the first-ever scientific workshop focused solely on GM-1 gangliosidosis (GM1) research for families and others with an interest in the disorder. GM-1 is a severe rare lysosomal storage disorder with a birth prevalence estimated at 1 per 200,000. Cynthia Tifft, M.D., Ph.D., Director of the Pediatric Undiagnosed Diseases Program at the National Human Genome Research Institute/ NIH, a preeminent GM-1 research scientist and clinician, will chair the workshop on Saturday, April 9, 2016. It is being held in conjunction with the National Tay-Sachs and Allied Diseases Association (NTSAD) 38th Annual Family Conference in Orlando, Florida.

Dr. Tifft will open the workshop with an overview presentation on GM-1. Expert scientists will then discuss the latest research and therapeutic approaches, including gene therapy, pharmacological chaperones, and enzyme replacement therapy.

A live broadcast will be openly available on NTSAD's YouTube channel and will be accessible through the NTSAD, Cure GM1 Foundation and Lysogene websites.

"We are very grateful for this opportunity to bring together those affected by GM-1 Gangliosidosis for this unique workshop," said Christine Waggoner, Cure GM-1 Foundation President "with so few biotechnology companies working on GM-1 treatments, Lysogene shines very brightly in the constellation of hope."

"NTSAD welcomes the opportunity to bring an additional dimension to our Annual Family Conference, and the addition of Cure GM-1 Foundation to our family," said Susan Kahn, Executive Director, NTSAD, "The more resources and attention we bring to all rare disease, the sooner we will see the realization of our hopes for successful new therapies for patients and their families."

The NTSAD Annual Family Conference is organised for families and individuals affected by Tay Sachs and related diseases, including GM1, to enable the sharing of experiences and information, to bring understanding, care and support and for the many families impacted by these rare genetic disorders.

Families and researchers interested in attending are invited to register for the Conference, which comprises sessions of support, experts and resources. Please contact the NTSAD office for more information.

About Cure GM1 Foundation
The Cure GM1 Foundation is dedicated to hope and to directly funding research for a cure for GM1 Gangliosidosis, a lysosomal storage disease that attacks the brain and spinal cord and is always fatal in children. GM1 is a progressive and degenerative condition with an extremely broad and debilitating array of symptoms and complications. This non-profit organization was founded by parents of children who suffer from GM1 who seek to save the lives of all those who suffer from this wretched condition.

About NTSAD
The nation's longest-standing rare disease advocacy organization, National Tay-Sachs & Allied Diseases Association (NTSAD), founded in 1957, funds research toward treatments and a cure for Tay-Sachs and related genetic neurodegenerative lysosomal storage diseases and leukodystrophies. NTSAD also provides comprehensive support to affected families worldwide. Having pioneered community education about carrier screening that became a model for all genetic diseases, NTSAD's education initiatives promote screening and prevention to the public and healthcare community. More information about NTSAD, Tay-Sachs, GM-1, Canavan, Sandhoff and related diseases is available at http://www.ntsad.org.

About Lysogene
Lysogene is a clinical stage biotechnology company pioneering in the basic research and clinical development of AAV gene therapy for CNS disorders with a high unmet medical need. Since 2009, Lysogene has established a unique platform and network, with lead products in Mucopolysaccharidosis Type A (Sanfilippo A) and GM1 Gangliosidosis, to become a global leader in orphan CNS diseases.

For more information www.lysogene.com.

CURE GM1 FOUNDATION

LYSOGENE

NTSAD

Information & Resources

Manchester Airport's Scheme for Children on the Autism Spectrum

Manchester Airport has introduced a scheme to help fast-track children with autism through security. Children with autism often find airports stressful and confusing, so this new initiative is designed to take some of the stress out of travelling.

The airport has promised that once parents can confirm that their child is on the autism spectrum they will provide a wristband that will allow the family to be fast-tracked. Booklets are also available to help parents plan the journey with their child.

For more information please visit http://www.manchesterairport.co.uk/about-us/media-centre/airport-awareness/

Challenging Behaviour Foundation

The Challenging Behaviour Foundation is a charity for those with severe learning disabilities and challenging behaviour. Their vision is for people suffering with these conditions to be given the same life opportunities as everyone else.

They aim to build awareness of these issues, empower families through information and training, and to help other organisations deal more effectively with those with severe learning disabilities and challenging behaviour.

For more information on the Challenging Behaviour Foundation, please visit www.challengingbehaviour.org.uk/

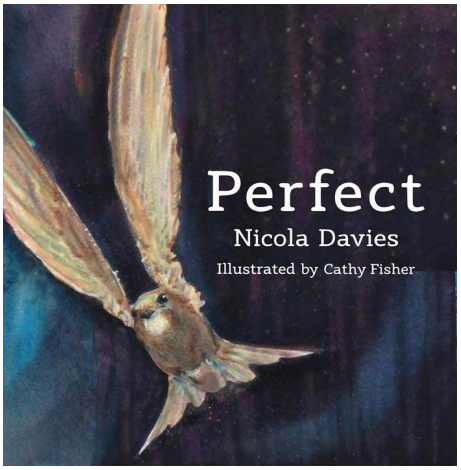
Naidex Event

This April the NEC in Birmingham will be hosting Naidex, the biggest disability, rehabilitation and homecare event in the UK. The event will showcase the products of over 200 exhibitors, which will include areas such as mobility, hoists, bathroom adaptations and multi-sensory environments to name but a few.

Exhibitions are not the only thing on offer at Naidex, there will also be an opportunity to attend a range of seminars.

Naidex will run from 26th April (which is available only for trade and healthcare professionals only) to 28th April (open to everyone).

For more details and to register your place please visit www.naidex.co.uk/.



Perfect by Nicola Davies

Children's author, Nicola Davies, has recently written a new book entitled *Perfect*, which tells the story of a young boy whose new baby sister is born with a disability. *Perfect* is his tale of coming to terms with her disability through his experience of the natural world.

The book is due to be released on 31st May 2016 by Graffeg Books, and promises to be a great way to open up communication about disabled siblings and the thoughts that children have on this subject.

Fleet Operations staff completed the infamous Tough Mudder as part of their fundraising for 2016 having chosen the MPS Society as one of their Charities of the Year. Read more on page 27.

Fundraising

Welcome to the fundraising section of the MPS Magazine, a section that is all about our members and supporters who raise the money and awareness that keeps us going.

April is already here and that means that MPS Awareness Day is approaching fast! Held on 15th May each year, this important day is when we ask everyone to get involved to help spread the word about MPS, Fabry and related diseases. Take a look at pages 38-40 to find out more about MPS Awareness Day and how you can join in.

Thank you to all the companies and organisations that have supported us, both this year and last. We have seen some great fundraising in the workplace, which is thanks to those who speak up and put our name forward. If the company you work for selects a Charity of the Year, why not ask them to consider the MPS Society? See page 36-37 for more information.

If you are at a loss as to how to start fundraising, take a look at our website (www.mpssociety.org.uk) or email fundraising@mpssociety.org.uk for your inspiring fundraising pack! (Hotmail users please remember to check your junk folder if emailing us, as quite often our replies end up in there!)

We still have some places available for the Great Manchester Run, Great South Run and British London 10K, so please do get in touch if you are interested in running for us. We ask for a minimum sponsorship of £200, but these events really do offer an unforgettable experience and our fundraising team are on hand to offer advice and resources to help you reach your target.

Elkie Riches
Fundraising & Information Officer

Taking on the Nippy Dipper

Each year, the Aberdeen Lion's Club organise The Nippy Dipper, an event held at Aberdeen beach at which several dozen hardy but clearly crazy people fling themselves into the freezing waters of the North Sea for fun and for charity. I was always one of those who looked at the pictures in the paper and shivered in sympathy, but in 2015, as I approached my fiftieth birthday, I decided that I wanted to challenge myself and give two fingers to the advancing years, so I took the plunge (do you see what I did there?!) and downloaded the application form. My daughter Kirsty agreed to join me and we decided we would dress up as Santa's Little Helpers.

Choosing which charity to fundraise for was a very straightforward decision, as my friend and colleague Nicola Edmond and her husband Bruce had had little Jack in April last year who was diagnosed with one of the life-limiting mucopolysaccharide associated conditions at 3 months old. Nicola and Bruce have a very supportive network of family and friends and have been nothing short of inspirational to all of us, I'm sure, as they have done as much as they possibly can with Jack to create fantastic family memories and experiences, including a trip to Pittodrie Stadium, the home of Aberdeen Football Club, and building sandcastles on the beach.

The Boxing Day Nippy Dipper sadly had to be cancelled due to the weather, but it went ahead a week later. The weather didn't exactly dawn bright and clear – more grey skies, wind and rolling waves – but Kirsty and I had already decided that 'come hell or high water' we were going in and we were joined by my cousin Richard who had come up for Hogmanay in Aberdeen, and foolishly volunteered himself!

"The three of us ran straight in, deciding it was a case of go big or go home"

The Lion's Club did a sterling job of wrangling all 91 of us safely across the road and into a line, facing the sea. Many were in fancy dress; I saw several tutus, a couple of minions, a colleague wore speedos and an apron featuring a kilt, at least one Santa and an elf, and a bear...and we counted down...and we were off!

The 3 of us ran straight in, deciding it was a case of go big or go home. Not for us a sedate paddle – oh no, Richard launched himself into a wave head first, Kirsty and I danced The Strictly, we gasped and screamed like wee girls as the waves hit and soaked us from head to toe and before we knew it, the time was up! First in and last out, but the time had flown by. I have never experienced an adrenalin rush like it; I have absolutely no memory of being cold, I just recall the sheer joy of doing something so crazy with people I love, supported and cheered on by friends and family, and in honour of a beautiful boy and his mummy and daddy.

We will definitely be signing up to do the Nippy Dipper again and I thoroughly recommend it as the best possible way to blow away your post-Christmas or New Year cobwebs, and raise some money for great causes. And as for being 50? It's fab-u-lous and I'm looking forward to my next challenge – Go Ape, a treetop adventure. I'm mortally terrified of heights, but after the Nippy Dipper I feel invincible!

Jane Tewnion

Our thanks to Jane, Kirsty and Richard for braving those icy waters and raising a brilliant £679!

Charity of the Year

Our thanks to HVPD and the Nuffield Hospital for supporting us in 2015, and to Fleet operations for choosing us for 2016!

HVPD

At the beginning of 2015 HVPD named the MPS Society as our Charity of the Year, and we have had a fantastic response from staff with the fundraising done this year.

"As well as wanting to raise awareness of the great work they do every day, we wanted to support the family of one of our colleagues at HVPD, whose granddaughter Gracie has been diagnosed with Morquio Syndrome, which affects less than 100 people in the UK, and only around 3,000 worldwide," said Kelly Thorley, HR Manager.

We have raised a total of £450 for the MPS Society and would like to thank everyone who has supported us through donations and taking part in our fundraisers!

HVPD

Our thanks to all staff at HVPD for getting involved in some excellent fundraising, which included a bake sale, Wear It Wicked (pictured above), Christmas Jumper Day and even a Man Vs. Food Challenge!

Thank you also to Senior Hardware Engineer, Chris Hopley, who put our name forward in support of his granddaughter, Gracie (MPSIV).

HVPD staff meeting Gracie!

Nuffield Hospital

My name is Joanna Wilson-Smale, I am 29 years old, I work as a senior radiographer for the Nuffield Hospital in Plymouth and I have MPS type I Scheie syndrome.

Every year the Nuffield Hospital Plymouth choose a charity to support for the year, with all the money from events held that year going to the charity. Last year I decided to put the MPS Society forward and they accepted.

There were many events held including quiz nights, a summer BBQ raffle, a Christmas raffle, and bake sales. The MPS Society collection pots were placed round the hospital, including by the various coffee machines and both patients and staff were incredibly generous. We were overwhelmed by the generosity of our patients and Nuffield supporters, including Drakes Jewellers in Plymouth who donated a Swarovski crystal necklace for our Christmas raffle.

I am so grateful to all who donated towards the MPS Society last year, but am also happy that the staff at the hospital now have a greater appreciation and understanding for MPS disorders. We raised an amazing £2,594, which we hope will make a difference in some way to the MPS Society.

Joanna Wilson-Smale

Thank you to Joanna and the staff at Nuffield Hospital for their wonderful support through 2015. As Joanna points out, raising awareness is also very important - the more people that know about MPS and related diseases, the better. If you would like to raise awareness and funds at work, don't forget to take a look at our website for some ideas, and get in touch by emailing fundraising@mpssociety.org.uk.

Fleet Operations

Here at Fleet Operations we manage bespoke fleet solutions for several major organisations including Heineken, Mitchells and Butlers and Bosch. We are a team of approx. 40 people based in Newcastle under Lyme.

This year our charity committee has selected three charities to fundraise for and the good news is that the MPS Society is one of them. After I asked if we could hold an MPS Awareness day in the office (as my daughter Emily Bradshaw suffers from MPSI Hurlers), the charity committee decided to adopt the MPS Society as one of their charities for 2016.

We will be holding several charity events throughout the year, raising much needed funds and of course spreading awareness. Some of the activities planned for the forthcoming year include BBQ Lunch, Climb up Snowdon, CycleLondonOrbital, Charity Golf Day, Tough Mudder Challenge, plus plenty of cake sales and dress down days.

Gayle Bradshaw
Mum to Emily Bradshaw, MPSI Hurler

Would you like to make the MPS Society your Charity of the Year?

Many businesses, large and small, choose a charity each year to be the lucky recipient of their all their annual fundraising. Quite often the charity is selected by a voting process where staff members are asked to put forward charities that are important to them, and these are then put in for a vote, the winner becoming the Charity of the Year.

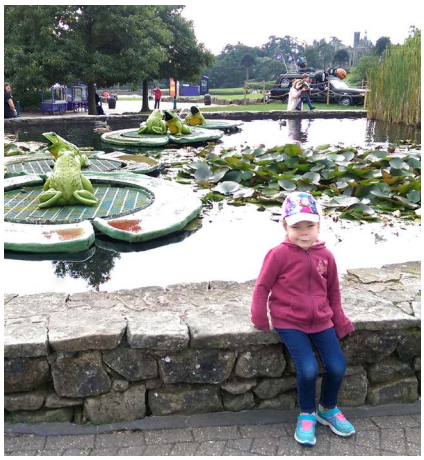
If your workplace runs a Charity of the Year scheme, why not suggest the MPS Society? It could be as simple as that to get the ball rolling. Not only would it make a difference to us by raising funds to help us to continue our work, but as you can see from the examples on these pages, it is also a great way to spread the word about MPS and related diseases among your colleagues, who will then talk to their families and friends about it, and before you know it you have raised a lot of awareness!

How do I fundraise at work?

As you can see from HVPD, Nuffield Hospital and Fleet Operations, there is no right or wrong way to fundraise at work. Bake sales, raffles and dress down/up days are some of the most popular ways to raise some money, but you can be as creative as you like. Whether you organise a challenge for a group of colleagues, such as the Tough Mudder, or whether you simply place one of our collection boxes in your cafeteria, it all helps. Fundraising is also brilliant for team-building and letting your hair down in the office!

What do I need to start?

If your workplace chooses us as their Charity of the Year (yay!) the best way to start is by letting us know - just email fundraising@mpssociety.org.uk or call 0345 389 9901. We can then send you out a fundraising pack, which is full of great fundraising ideas to help you on your way. Don't forget to take a look at our website for more information and inspiration: www.mpssociety.org.uk.



Emily during her recent trip to Alton Towers!

MPS AWARENESS DAY 15th MAY 2016

WE NEED YOU!

MPS Awareness Day is a fantastic opportunity to help us to get MPS and related diseases noticed! Every year we call on all our members and supporters to get involved by either Wearing it Blue (see below!), holding your own fundraiser, taking to social media to post and tweet, or just talking to friends, family and colleagues about MPS and why our work is so important. We want to make the day bigger than ever so that we can reach more people..and we need your help to do it!

WEAR IT BLUE!

Show your support for those affected by MPS diseases and Wear it Blue to mark MPS Awareness Day. This is a great way to get your friends, colleagues, family and community involved, and it couldn't be easier: just ask everyone to wear blue for the day and make a donation! A quick whip-round at the office, nursery or classroom can raise a surprising amount of money and is an effective way of opening up a conversation about these rare but devastating diseases.

Request a fundraising pack by emailing fundraising@mpssociety.org.uk and don't forget to post your Wear it Blue selfies to our facebook page or tweet @MPSSocietyUK #MPSAwarenessDay #WearitBlue2016!

WHY HAVE MPS AWARENESS DAY?

If you have been affected by MPS or a related diseases, you will no doubt be well-versed in explaining what it is and how it effects you or your child or loved one. These diseases are rare and sadly the general public are largely unaware of the challenges and devastation these conditions can cause. MPS Awareness Day is a move to address that: raising awareness among the general public and among medical professionals can really help families to feel understood and get the support they need.

MPS Awareness Day is also a day to celebrate those who are living with MPS, and those who have lost their lives to it, and to mark the achievements that have been made and those yet to come.

MPS AWARENESS DAY FAMILY EVENT
GULLIVER'S FAMILY DAY, SUNDAY 15TH MAY 2016

To mark MPS Awareness Day 2016 on Sunday 15th May we are holding a family day at Gulliver's Land Theme Park in Milton Keynes.

We are inviting sufferers, siblings and their families to come and enjoy the theme park and a buffet lunch in celebration of this special day.

Those attending will have the opportunity to experience the fun and excitement of Gulliver's range of rides and shows, most of which are wheelchair accessible, including the Runaway Train, Carousel, Lilliput Woods Theatre, Gully Mouse's Magical Castle, Dodgems, Crazy Barrels, Tree Top Swings and Flying Carpet.

The booking form will be enclosed along with our Spring 2016 MPS Magazine, but if you need a form sent to you, please get in touch by calling 0345 389 9901 or email mps@mpssociety.org.uk.

Gulliver's Theme Park is perfect for families with children aged 2-13 years old, and promises a great way to mark MPS Awareness Day. For more information and to book your place, please see the booking form enclosed with this magazine.

See you there!

TAKE TO THE SKIES FOR MPS AWARENESS DAY!

This year pharmaceutical company, Shire, have got together with the MPS Society and the International MPS Network to come up with a brand new campaign for MPS Awareness Day, which is all about connecting MPS patients around the globe.

The campaign, which runs from 15th May to 15th June, will offer a fun and exciting way to raise awareness and connect with others to mark this important day.

Keep your eyes on the campaign's official website - www.mpsday.com - which will be taking off in May with lots more information on how you can join in!

Please turn the page for more information on this exciting new campaign to mark International MPS Awareness Day!

Keep up to date with what's going on with MPS Awareness Day 2016, and let us know what you are doing to celebrate the day by following us on Facebook and Twitter, or by dropping us an email:

facebook.com/mpssociety MPSSocietyUK fundraising@mpssociety.org.uk

TAKE TO THE SKIES FOR...

MPS AWARENESS DAY 15TH MAY 2016

TAKE TO THE SKIES!

15TH MAY - 15TH JUNE 2016

Help us connect MPS patients around the world by sharing your customised plane with your friends

SHOW YOUR SUPPORT!
BUILD AND FLY YOUR PLANE
SHARE YOUR PIC

HELP US REACH OUR TARGET OF 20,000,000 AIR MILES

& 8,000 CONNECTIONS

COMING IN MAY! WWW.MPSDAY.COM

Take to the Skies campaign supported and funded by Shire

SPREAD THE WORD ON MPS AWARENESS DAY WITH SOME OF OUR GREAT MPS SOCIETY MERCHANDISE

MPS Society T-Shirt - €5

Wear it loud and proud as part of your fundraising event, or just to mark the day.

Available in a range of sizes in either a men's or women's t-shirt.

Pin Badge - €1.50

Measuring at just 2.5cm across, these enamel and metal pin badges can be worn discreetly on your lapel or bag to show your support (also great for wedding favours!).

Awareness Ribbon - 50p

Wear It Blue for MPS Awareness Day with one of our ribbons! Buy some for the office to get colleagues involved and really raise some awareness!

Shopping Bag - €3.50

Support the MPS Society while you shop with this eco-friendly alternative to plastic bags. Lasting, durable and a great conversation starter!

Photo/Memo Holder - €3

Ideal for your desk to hold photos of loved ones or notes to keep you organised.

Visit our online shop at www.mpssociety.org.uk for more merchandise, including button badges, teddy bears, notelets and wristbands!

The Princess & the Peastalk Fundraising Panto

What do you get if you cross The Princess and the Pea and Jack and the Beanstalk?

The Princess and the Peastalk, obviously, oh and quite a few pea-related puns!

The mix of these two classic fairytales was the premise for the Barnwood Amateur Dramatics Company's (BADCo) panto this year. Thanks to the Eaton family, whose sons' Archie and Isaac are both living with Morquio, the MPS Society were invited along to a full performance.

The panto was Anna Eaton's debut as writer and director and incorporated all the expected puns, songs and "he's behind yous" you'd want from a proper English panto. With classics such as Reach for the stars and Mamma Mia it was difficult not to jump up on stage and join in. A special mention has to go to Archie Eaton for his superb role as narrator and best supporting actor to Isaac Eaton as the Deputy Court Jester. With a cameo from their dad, Dave Eaton, it really was a family affair.

The play followed the exploits of Princess Lily who lost her crown and cape whilst sleeping in the forest and ended up seeking shelter in a nearby palace, interrupting the Prince's birthday party in the process. Unable to prove she's a princess without her crown, fun and frolics ensue and even a witch, a talent contest and a giant peastalk make it into the show.

BADCo has been running for over 20 years and the team effort put in to make this performance a success was remarkable. It makes us at the MPS Society especially proud to see two of our young members steal the show!

And as if an invite to the show itself wasn't enough, the panto also raised over £1000 each for the MPS Society and the Restricted Growth Association. BADCo always donate their profit from productions to charity and to date they have raised well over £9000!

If you would like to be kept up to date with BADCo's news and activities visit www.badco.org.uk.

For more information about the Restricted Growth Association visit www.restrictedgrowth.co.uk.

Fundraising for Evie

The Skills and Training is a local training provider based in Port Talbot which has been delivering comprehensive training and learning programmes since 1980. It has been working in partnership with the Princes Trust for over twenty five years to deliver the Team programme and in 2015 was awarded "Best Delivery Partner in Wales" by the trust. In January 2016, Skills & Training celebrated having run 150 Princes Trust teams, assisting over 2000 young people on this programme alone.

As Part of the 12 week Princes Trust Team programme the team is required to undertake a team challenge, which involves being responsible for choosing an individual or organisation to raise funds for, taking the individual out on a day trip or completing an activity to give something back. The team are also responsible for identifying a suitable team challenge, undertaking fundraising and then celebrating their achievements with the individual or organisation.

The team decided to raise funds for a local young lady called Evie Wiggins who they had been made aware as Skills & Training had chosen the MPS Society as their charity of the month to raise funds for. Evie and her family have been supported by the MPS Society as Evie suffers from a rare genetic condition called Sanfilippo Syndrome and the team was touched by her and her family's story. The young people in the team were keen to undertake fundraising to make her Christmas special. The team organised a Christmas themed sponsored walk to raise funds so they could then invite Evie to attend Skills & Training for a tea party, meet Santa and receive some gifts from the money they had raised from their team challenge.

Evie attended Skills & Training and was met by the team and Santa who gave Evie her gifts. Evie and her family were overwhelmed by the generosity and a great afternoon was had by all.

Following on from the Princes Trust team challenge it was decided by the staff at Skills & Training to run a raffle on the evening of the Princes Trust 150th Team's presentation and learner awards evening which was held at Margam Orangery on 14th January 2016. Staff contacted local businesses and were overwhelmed by the amount of items that were donated by businesses and individuals for raffle prizes.

Raffle tickets were sold on the night to the 200+ guests that attended. These distinguished guests included the Leader and Deputy Leader of Neath Port Talbot Council, Chief Executive of Neath Port Talbot Council, Mayor of Neath, local councillors, Director of the Queens Trust Nicola Brentnall, Welsh International Rugby and Ospreys player Dan Lydiate and Swans Football Ambassador Lee Trundle. Entertainment for the evening was provided by local singer-songwriter John Nicholas who had been supported by the Princes Trust to allow him to achieve his dream and release his first song and embark on a European tour.

The evening was a celebration not only of the personal achievements of the Princes Trust learners but also recognising the achievements of the learners that have attended various training programmes offered by Skills & Training.

At the end of the evening the grand total was announced: £630 had been raised for the MPS Society. Evie and her Mum took to the stage to receive the cheque on behalf of the MPS Society.

Skills & Training NPTCBC

Fundraising for Archie

On the 19th of Dec TIC held a fun day in support of MPS.

TIC are a Telecoms solution provider based in Wolverhampton and they decided to support the charity as Marketing Director, Sean Pearson's nephew Archie has MPS.

During the day at the TIC office in Wolverhampton there were various events including silent auctions for Christmas related gifts, days off from work as well as Sean having his legs waxed!

A great combined effort resulted in raising £828.95 on the day

Well done everyone!!

Sean Pearson
TIC

Haddenham Mummers

Long-term supporters of the MPS Society, the Haddenham Mummers, did a fantastic job of raising funds for good causes in the run-up to Christmas 2015. The group raised £510 in support of MPS, with further donations going to other charities, totalling £2,000.

Jenny Hardy kindly represented us at the cheque presentation (pictured above, courtesy of www.haddenham.net).

The Mummers spend the festive season performing traditional folk plays in the local area.

Our thanks to all involved.

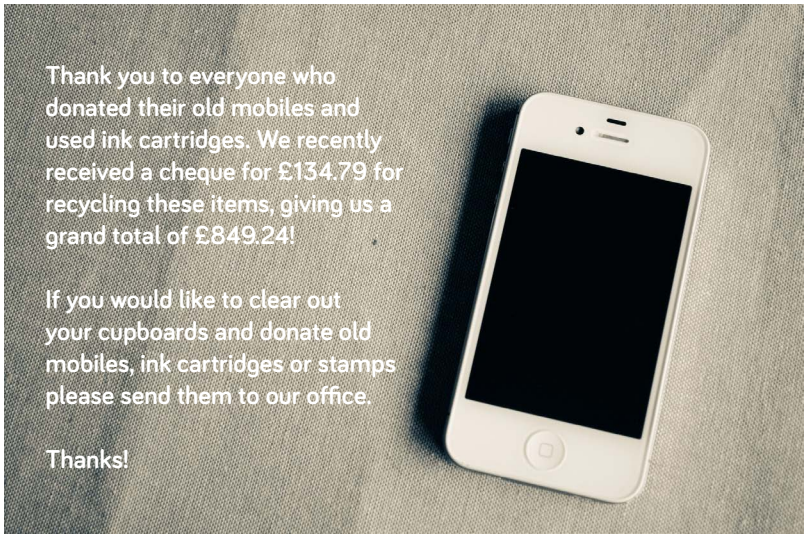
GSK Volunteers

Back in October of last year, pharmaceutical company GlaxoSmithKline got in touch to ask whether they could volunteer for us as part of their Orange Day, which marks the company's day of service.

We were delighted to invite the team to our office to help pack our Winter 2015 MPS Magazine, a huge task that is usually the work of MPS staff, so we were very grateful for the assistance!

The GSK team made a great job of it, getting nearly all the magazines packed and ready to post in a morning, and they have planned to return to help with future magazines, which is a huge help to the MPS Society.

Our thanks to Barbara, Martin, Angela, Paula, Nina and Mike from GlaxoSmithKline for all their hard work!



Legacies

Making a Will and how leaving a legacy to charity in your Will can benefit both the charity and your friends and family

It's important to make a Will, whatever your circumstances. The one certain thing in life is that we will all die at a certain point! Dying without a Will, known as being "intestate", means your estate (which means your money, possessions and property) might not go to the people you would otherwise want it to and it can create problems for those left behind.

Leaving a legacy

In most cases, the first priority in a Will is providing for family and friends. However, many people also leave gifts to charities, known as legacies, in their Wills.

Legacies are a really important source of income for charities, especially the MPS Society where we rely on donations and fundraising to keep providing our vital services of Support, Research and Advocacy. We have a legacies section on our website where you'll find all the necessary information, including our Charity registration number etc. Otherwise it's as straightforward as including a simple sentence in your Will.

Types of legacy

Legacies can be divided into three categories:

• A fixed sum of money (Pecuniary)
This may seem like a simple option but bear in mind that the effects of inflation could mean that the true value of this gift could become less than you intended over time. To plan for this, either update your Will regularly or link your legacy amount with inflation.

• A percentage of the net value of the estate (Residuary)
A share of your residuary Estate means you can leave a set proportion of your residuary Estate to charity after all other funeral debts and testamentary expenses including pecuniary legacies (above) have been settled.

• Specific Legacies
This could be anything, but typically land, property, shares or personal possessions.

If you have already made a Will but now want to leave a legacy to the MPS Society, or any other charity, you can make an addition or change it without having to rewrite your current Will. This addition is called a Codicil and a Solicitor can advise on how to add this to an existing Will.

Tax benefits

As well as helping a good cause, charitable bequests also have financial benefits for your friends and family.

If you leave a gift to a charity in your Will, the value of the gift will be deducted from your estate before inheritance tax (IHT) is calculated. In some cases leaving a charitable bequest may help reduce the total value of your estate below the taxable threshold.

Further it is possible for the IHT to be reduced to 36% (a 4% reduction from the otherwise 40% tax rate) if 10% or more of an estate is left to charity. Taking advantage of this reduced rate would likely require advice from a Solicitor. This is worth considering, especially if your estate exceeds the taxable threshold.

By Jessica Reid
Trustee and Qualified Solicitor (practising in Family Law)

Thank you to all our donors including...

Emma Beckham held a 'Pirates and Pyjama Party' at her children's group and raised a brilliant £360.

Karen and Andrew Weedall sold Webb and Ivory Christmas gifts to friends, family and work colleagues, earning £43 of commission, which they kindly donated to the MPS Society.

Pat Gardner recently celebrated her 70th birthday and asked her family and friends to donate to the Society in lieu of buying presents. Pat and 8 members of her family suffer from Fabry disease.

LG Optical in East Sussex raised £192.50 by raffling a hamper full of Christmas goodies.

Robert Kenton and partner Jenny raised £79.50 by performing Christmas tunes for their local walking group. Robert played the snare drum and Jenny played the accordion.

Hefin and Bethan Richards sent in a further £75, in addition to the £620 they had previously donated in lieu of wedding presents. Our thanks to the happy couple!

Chris Smeaton and Abbas Al-Mamouri slept rough in Birmingham over Christmas to raise funds and awareness for three charities, one of which was the MPS Society. Chris and Abbas did really well and raised £1625.42 for us.

Sean Hannah did the Movember Challenge and raised £25.

Zeenat Begum held a charity dinner and raised an amazing £382.14 in support of her son who suffers from Morquio.

Students at Clifton with Rawlcliffe Primary School collected donations after their Christmas productions and raised a fantastic £421.97.

Katy Brown and family held a celebration party to mark NHS England's decision to fund MPSIVA drug, Vimizim, raising £126.90 in donations.

The Old Steine Lodge held a charity raffle and raised £105 for the MPS Society.

Rowdeford School raised a brilliant £150 for the MPS Society at their annual carol service.

The Bournemouth Ladies' Festival Association raised £550 by holding a raffle and auction at the Brentford Masonic Lodge's Ladies Festival in November 2015.

Emma Hiller sent in a further £85 toward the sponsored walk she organised and completed last May, giving her a grand total of £1,145.63!

All the used stamps that you send in have raised a brilliant £50, keep them coming please!

The children at 'Stars' playgroup for disabled children in Coventry paid £1 to wear a Christmas jumper and raised £50 for the MPS Society in memory of Daniel. Thanks to Rash (who works at 'Stars' on a Saturday) and Sandra Singh for sending in the money.

The Lodge of St John 191, Bury raised £1,925 for the MPS Society on behalf of Emma Slater who suffers from Hurler disease.

The Haddenham Mummers performed a traditional village mummer's play in pubs around Haddenham in the days leading up to Christmas and collected money after each performance to share between local good causes, this included £510 for the MPS Society.

Kim Palmer joined in with PubAid's "World's Biggest Pub Quiz" at The Eastfield Hotel in Portsmouth, organising an exciting event that included face painting and selling MPS Society goodies. She raised a wonderful £488.16 in memory of Anabelle Shepherd.

Zeina McMillen, an employee of Regatta outdoor clothing, and her fellow Regatta Yogis used their yoga classes as a platform for giving back to a good cause and kindly donated £277.20.

Mr Wilson raffled off his 65th birthday cake, which was in the shape of a ukulele, and raised £40 amongst Thornton Le Dale Ukuleles music group and residents of Roxby Gardens.

The Highcrest Academy in High Wycombe raised a superb £1078.49 on a non-school uniform day.

David Forrester and the rest of his team, Sighthill North, raised £310.50 at a recent dress down day which was matched by his employer Lloyds Banking Group making a grand total of £621.00! David donated to the MPS Society on behalf or Amanda and Darren Scott whose daughter Sophia has Sanfilippo.

Kath Hiller raised £155.43 by collecting donations at a Christmas Tree Festival.

The Year 2 classes of **James Elliman Academy** in Slough have been busy fundraising and donated a total of £39.74.

Thanks to **Paul Shields** for donating his car to Giveacar.co.uk and making £29.40 for MPS!

Hannah Macey collected £55.61 in change and foreign and out of date currency around the house after her late grandmother, Shirley Macey passed away.

Donations

Kate Hughes; staff at the Central Policy Secretariat of the UK Statistics Authority; Terry & Irene Watts; J A Pye Settlement; Keith Coombs Trust, Bellway plc, Reuben Foundation; The Vodaphone Foundation; The City Bridge Trust; Carrington Charitable Trust; Miss E Jenkins; ShareGift; Mrs D E Peirson; Mrs A Baker; Sarah Agar; Simon Anning; A J Cumming; June Gilbert; Emma Hudson; Heidi Hunter; Hugh Johnson; Dr A. & Mrs N. Bansal; Peter & Andrea Lewis; Andy & Jenny Hardy; Mrs Joan Doyle; Mrs R.J. Scott; Mr & Mrs A. Eaton; Colin & Dorothy Robinson; Mike Southwell; Richard & Kay Dunn; F Robinson; The Bernard Sunley Charitable Foundation; Gary Harlock; John Moore; Michelle Stark; Jacqueline Best; Alan & Monica Bowen; Fiona Scott-Burns; Florence Turner Trust; David Rawlins

In Memory

Maureen Shelmerdine; Colin Arrowsmith; Joan Colvin; Dorothy Caroline Mary Parfitt; Lady Shauna Gosling; Darren Horsley

Collection boxes, stamps, foreign coins, mobile phones, ink cartridges, jewellery

Andrew Culley; Christopher Croft's family; Langlea House Care Home; Karen & Andrew Weedall; Sally James; Ian Evans & Arriva The Shires; Claire & Bob Stevens; Wilma Robins; Arlene Murray; Beryl country house; David Fowler; Anabelle Shepherd

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

N Cadman; J Wilson; A Tresidder; E Cox; Mr Thompson; K Osborne; M Rigby; Stuart Robinson; M Peach; C Garthwaite; Raymond Arnold; J Ellis; I & V Pearson; D & S Peach; J Daligan; M Malcolm; E Mee; S & D Greening; Mr Hahner; E Moody; K Brown; Z Gul; E Brock; M Fullalove; M Leask; M Reeves; E Parkinson; G Ferrier; R Taylor; R Gregory; L Stillwell; R Henshell; K Bown; S & J Home; V Little; J Winzar; J Casey; E Lee; K & J Hudson; D Winzar; J & V Hastings; R & K Dunn; C & M Gibbs; Mr & Mrs Cock; Alan Dickerson; M Kalsi; P Summerton; S Littledyke; N Saville; M Tosland; C Cullen; S Brown; V Lucas; D Forbes; G Simpson; S Winzar; P & R Shrimpton; W Cavanagh; Barbara Harriss; L Brodie; A Sabin; A Ephraim; J Garthwaite; Paul Berg; Abby Thomas; Elizabeth Merryweather; Michael Morris; Tim Peach; Nick Miles; Peter Rennoldson; Amanda Laycock; Matt Mould; Tmara Senior.

Thank you also to all those who donated anonymously - we don't know who you are, but we think you're great!

WHAT IS MPS?
MPS stands for Mucopolysaccharide, which along with Fabry and other related diseases, are a group of rare genetic conditions that cause progressive, life-limiting disability.

Wear It Blue!

FOR MPS AWARENESS DAY

Sunday 15th May 2016

IT'S EASY TO JOIN IN - JUST WEAR BLUE & MAKE A DONATION!

- Text MPSS01 £2/£5/£10 to 70070
- Visit WWW.MPSSOCIETY.ORG.UK
- Call 0345 389 9901
- Or hand your donation to the event organiser

WHO AM I DONATING TO?
The MPS Society is the only UK charity providing support to children, adults and their families, who are affected by these devastating diseases. For more information please visit www.mpssociety.org.uk

Registered office at MPS House, Repton Place, White Lion Road, Amersham, Buckinghamshire, HP7 9LP, UK
Registered Charity No. 1143472. Registered as a Charity in Scotland No. SCO41012
Registered as a Company limited by guarantee in England & Wales No. 7726882