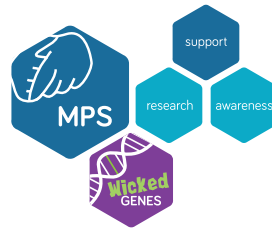


# THE MPS MAGAZINE



**Society for  
Mucopolysaccharide  
Diseases**

**Support  
Research  
Awareness**

**Winter 2015**

[www.mpsociety.org.uk](http://www.mpsociety.org.uk)

### *It's a yes for Vimizim!*

NHS England have announced that they will be funding the MPSIVA drug, Vimizim.

Read more about this decision and the Managed Access Agreement on page 5.

### *14th International Symposium*

Due to be held on 14th - 17th July 2016 in Bonn, Germany, the 14th International Symposium on MPS & Related Diseases promises a packed and informative programme.

If you would like to find out more, turn to pages 22 - 23

### *Skydive for MPS!*

Gary Harlock, with a group of friends, recently undertook a skydive to raise money for MPSII gene therapy. Read more about how they did on page 31



Good fun was had by all at Gulliver's Theme Park, thanks to funding from BBC Children in Need.

Read more on page 21

Front cover photo: Fun at Gulliver's Theme Park

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magazine@mppsociety.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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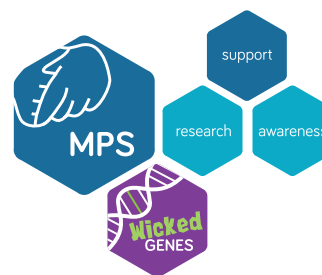
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## MPS Awareness Day 2016...

Join us in celebrating MPS Awareness Day on Sunday 15th May 2016, and help us raise awareness and funds to help support those living with MPS & related diseases. MPS Awareness Day is also a day to celebrate the treatments now available and those we hope are yet to come, and remember those we have lost.

Every year our supporters really get behind our Wear It Blue campaign and make the day their own, so although it may be early, put it in your diary and get ready to help us mark this special day!



### 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

### 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.

If you would prefer to receive your MPS Magazine by email rather than through the post, or if you would like to be taken off our mailing list, please let us know by calling 0345 389 9901 or emailing magazine@mppsociety.org.uk.

# Contents

4 × Chief Executive's Report

5 × Vimizim

6 × Governance and What's On

8 × Announcements

9 × Advocacy

14 × Your Stories

18 × Events

25 × International

26 × Research and Treatments

28 × Information & Resources

29 × Fundraising



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[twitter.com/MPSSocietyUK](https://twitter.com/MPSSocietyUK)

## Welcome

This edition of the MPS Magazine contains a great selection of topics, from your fundraising stories to advocacy news.

As many of you will be aware, NHS England announced that they would be funding the MPSIVA drug Vimizim (otherwise known as elosulfase alfa), and we are of course delighted by this result. Read Charlotte's article on page 5.

There are some fantastic events planned for 2016, including the 14th International Symposium on MPS and Related Diseases, which is to be held in Bonn, Germany on 14th - 17th July. Read more about this four day conference on pages 22 -23 and please also take a look at the programme, which we have enclosed with this magazine.

Please remember that if you have a story that you would like to share, email us at [magazine@mpssociety.org.uk](mailto:magazine@mpssociety.org.uk).

Happy New Year!

*The MPS Team*

*Visit our online shop*

**[www.mpssociety.org.uk](http://www.mpssociety.org.uk)**

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







## Chief Executive's Report

*Christine Lavery*

As we welcome in another New Year I, on behalf of the Trustees and staff team, want to wish all our members, their families and all the MPS Society's supporters our best wishes for 2016. We recognise that so many of our members have been dealt many challenges this past year but I want to assure you that our support service, strengthened by Louise Cleary who will be joining the Advocacy Support Team in January, are there for you. No request for help is too small or too onerous.

We have been delighted to see so many of you at our events over the past year. The Manchester and Liverpool Christmas parties were so well attended and, if the children's reactions were anything to go by, they loved the pantomimes. Equally, Christmas at Gulliver's in Milton Keynes two days before Christmas was a great hit. For the coming year we have other exciting events and activities planned, including taking some of our members and their families to the International Symposium on Mucopolysaccharide Diseases in Bonn, Germany in July 2016. If you are interested in this amazing opportunity please don't delay sending in the booking form enclosed with this magazine.

Research and education are important aspects of the Society's work and on the 9 – 10 April we are holding an Expert and Patient Meeting on Mucopolipidiosis at the Hilton Hotel, Northampton. This meeting with member childcare facilities is a unique opportunity for families to learn more about clinical management and research whilst the clinicians learn the patient experience. We recognise that many members' children have lost their lives to ML. If you would like to participate in this meeting, or just pop by, or maybe join us for dinner on Friday night, please do let us know.

I can't conclude without mentioning the terrific feat through joint working between the MPS Society, Prof Chris Hendriksz, BioMarin, NHS England and NICE in achieving reimbursement of Elosulfase Alfa (Vimizim) through a Managed Access Agreement announced by NICE on the 16 December 2015. This deal was hard fought but a triumph for common sense, equity and forging a pathway for current and future access to treatment decisions.

*Christine Lavery*  
*Group Chief Executive*





## Vimizim- it's a YES!

**If we could stand on the roof of MPS House and let off celebratory flares that's exactly what we would do to mark the significant progress made in our campaign for access to Vimizim.**

To recap, after the second committee meeting NICE released draft guidance that they were now minded to a positive decision if there was a Managed Access Agreement for patients in England.

A Managed Access Agreement (MAA) is a completely new, never been done before agreement which needed to be signed up to by NHS England, NICE, clinicians, the pharmaceutical company, and us, the patient organisation. Not an easy feat especially under very tight timescales. It was made perfectly clear that we either pulled together a Managed Access Agreement or had a 'no' decision from NICE.

A 'no' was definitely not an option. Through incredibly hard work by all concerned and a lot of dialogue, the deadline was met and a MAA was signed by NICE, NHS England, Professor Chris Hendriksz and Christine Lavery. On the 23rd November 2015, NICE released their draft final guidance that they had accepted the MAA and, providing there were no appeals, then a final positive decision would be announced on 16th December 2015.

This was indeed a fortuitous date as it came a year to the day that NHS England were to make a decision about interim access to Vimizim, but their process was of course found to be discriminatory against ultra-rare diseases.

What a year of huge emotional turmoil it has been.

NHS England have agreed that funding for treatment will be made available immediately, as this normally can take up to three months.

There has been so much emphasis on NICE and NHS England's decision as there are so many other countries which look to NICE to help guide their own decision making, including Wales, Northern Ireland and the Netherlands.

The MAA has both start and stop criteria which will determine access to treatment. The criteria was based on knowledge gained from the clinical trial. However, this is a first and whilst we are hugely relieved at the positive decision we will be keeping a close eye on whether the criteria is fair and we ask for regular communication with the Society and your clinician.

In Scotland, whilst the decision was a 'No' on an individual basis, all four children, one of which was not on the clinical trial, have had access to Vimizim approved, which is fantastic. If you live in Scotland and want to find out more about treatment please do not hesitate to contact the Society.

We have met with the Welsh decision makers and are working closely with them and are hopeful for a decision in early 2016.

Our Irish Advocate Alison Wilson is liaising with the Commissioners and she will be keeping us all up to date with progress in Northern Ireland.

*Charlotte Roberts  
Communications Officer*

# 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 4-5 September 2015 at MPS House, Amersham.

## Governance

The Chairman reported that she is still waiting for a completed self-appraisal form from one of the Trustees. The Group CEO distributed a paper on the 'Case for appraising both the Board and individual trustees'. The Chair reminded Trustees that if a Trustee misses three successive meetings the individual must consider their position. The skill base of the Trustees was discussed and it was agreed that through the MPS Magazine new Trustees with skills in accountancy and HR are sought. The Society's Strategic Plan was agreed subject to some grammatical amendments.

## Annual General Meeting (AGM)

The Minutes of the AGM were agreed and signed

Proposed	Jessica Reid
Seconded	Judith Evans

## Financial Management

The consolidated budget for 2015 was circulated previously and reviewed. Trustees liked Trustee. James Garthwaite's income and expenditure sheet which is a condensed version of the document the Group Finance Officer provided. The consolidated budget for 2016 was considered and approved.

## Personnel

The Group CEO confirmed that the post of Assistant Finance Officer has been filled.

The Trustees considered the proposal that the Finance Officer's title be changed to Group Finance Officer and this was duly agreed.

## MPS Commercial

The Group CEO gave an overview of the clinical trials that the MPS Pact team (Patient Access to Clinical Trials) were currently providing logistics for.

## Policies

Tim Summerton stated that he is also waiting for three Trustees to respond to the Financial Controls Policy Checklist. It was agreed that the Group CEO and Group Finance Officer prepare the

answers and the matter be deferred to November 2015.

## Advocacy Support

The Group CEO confirmed that two family Days in Manchester and Liverpool have been arranged and bookings were coming in. It was confirmed the ML Expert Meeting and Family Weekend will take place at the Hilton Hotel, Northampton after quotes for Alton Towers were too expensive for our budget. It was agreed that the Group CEO and Trustee, Wilma Robins, lead the planting at the Childhood Wood in October.

## Access to Medicines

Charlotte Roberts gave a compelling presentation to the Board on the 16 month campaign to achieve reimbursement of Vimizim in England and Scotland. Charlotte explained the rationale for the Vimizim Campaign from a political and press perspective. One of the key successes was having Katy and Sam Brown and about 15 other very active members / parents. The Board discussed the NICE process and Charlotte explained how we got from a 'NO' to a Management Access Agreement and a 'YES'.

## What's On

### MPS Regional Clinics 2016

#### MPSI - GOSH

12th January • 12th April  
26th July

#### MPSIII - GOSH

23rd February • 28th June  
13th September

#### MPSIV - GOSH

22nd March • 12th July  
8th November

#### Fabry - GOSH

26th April

#### MPS - BCH

5th February • 10th June  
16th September • 25th November

#### MPS Transition clinic - BCH

25th April

#### Fabry - BCH

27th May • 28th October

#### Adult Fabry - QE, Birmingham

9th February • 8th March • 12th April  
10th May • 14th June • 12th July  
9th August • 13th September  
11th October • 8th November  
13th December

#### MPSI Post HSCT (over 6 years) - RMCH

22nd January • 6th May  
1st July • 7th October

#### MPSI Post HSCT (under 6 years) - RMCH

29th January • 8th April  
8th July • 14th October

### Conferences and Regional Events

#### Scottish Information Day - Beardmore Hotel, Glasgow

20th January 2016

#### Welsh Information Day - Holiday Inn, Cardiff (North M4)

28th January 2016

#### Expert & Patient Meeting on Mucopolidosis - Hilton, Northampton

9th - 10th April 2016

#### MPS Awareness Day

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





## Up & Coming Events for 2016

### *Scottish Information Day - 20th January / Welsh Information Day - 28th January*

Our information days offer individuals, parents, partners, carers and professionals a wonderful opportunity to learn more from our series of presentations by experts in the field. These events also offer a chance for families and professionals to meet together to share their experiences and knowledge.

With topics covering areas such as patient management, access to treatment, education and prenatal diagnosis, the talks offering a range of useful and up to date information on MPS, Fabry and related diseases.

Booking forms for these events are available from our website or please contact the office if you need further information.

### *Expert & Patient Meeting on Mucopolysaccharidosis - 9th - 10th April*

The MPS Society is holding an expert and patient meeting, spanning Saturday 9th April to Sunday 10th April, that is dedicated to the sharing of information on Mucopolysaccharidosis.

Our programme contains a variety of talks that cover members' personal experiences, the latest therapies and research developments, and support and advocacy information. The programme also offers the unique opportunity for delegates to meet and talk informally, and for individuals, parents, partners and carers to share their own experiences with others.

To help everyone make the most of the weekend, children and vulnerable adults suffering from one of the Mucopolysaccharidosis and their siblings, aged 17 and under, will have their own social programme.

Booking forms will be sent out in the post. If you would like to be added to our mailing list for this event please email [mps@mpssociety.org.uk](mailto:mps@mpssociety.org.uk) or call 0345 389 9901.

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

Some of you may remember our coverage of the 13th International Symposium, which was held in Bahia, Brazil, where delegates enjoyed a packed programme from experts and families, hailing from all corners of the globe. This year the International Symposium is to be held in Germany in the dynamic city of Bonn, and it promises talks from world-renowned professionals, featuring updates on cutting-edge therapies and clinical trials, in addition to in depth presentations on aspects of care.

Children and youngsters will also have their own programme to keep them thoroughly entertained for the duration! There is also a MPS adult social programme.

For more information please see pages 22 - 23 and the enclosed programme.



## Announcements



### *Congratulations, Katrina!*

Cohen was born on 30th October 2015 to proud mum, and MPS Society Trustee, Katrina Fanneran.

Congratulations to Katrina and partner Kevin Mullins.

### *Happy 18th Birthday, Aisha!*



Back in November Aisha Seedat celebrated her 18th birthday. According to Aisha, she was planning on celebrating her 18th in style - as is only right, we think!

Aisha and her family have long been fantastic supporters of the MPS Society, and Aisha herself is one of our Young Ambassadors.

Happy Birthday from everyone at MPS House!

### *New Members:*

**Mr Hajou and Mrs Alrawi** have recently been in contact with the Society. Their daughter Mays (16 years) and son Wahid (11 years) have a diagnosis of Sanfilippo disease, Type C. The family live in the North of England.

**Mr Malik** has recently been in contact with the Society. His daughter has a diagnosis of Sanfilippo disease. Tabeer is 5 years old. The family live in the South East of England.

**Susanne** has recently been in contact with the Society. She has a diagnosis of Fabry disease. The family live in South Yorkshire.

**Stuart** has recently been in contact with the Society. He has a diagnosis of LAL D. He is 49 years old. He lives in the North East.

**Mrs Barbara Newson** has recently been in contact with the Society. Her son has a diagnosis of MPSIIIA. Sidney is 19 months old and the family live in the North East.

**Nicole** has recently been in contact with the Society. She has a diagnosis of LAL D. She is 23 years old and lives in the South West.

**Onaissa & Raissa** have recently been in contact with the Society. They have a diagnosis of Alpha Mannosidosis. The family live in the East Midlands area.

**Mr Khan** has recently been in contact with the Society. His son has a diagnosis of Sanfilippo disease. Muhammad is 6 years old. The family live in the North West of England.

**Helen & Shaun Marsden** have recently been in contact with the Society. Their son has a diagnosis of LAL D disease. Connor is 8 years old. The family live in the North East of England.

# 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@mpsociety.org.uk](mailto:advocacy@mpsociety.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.mpsociety.org.uk](http://www.mpsociety.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.

## Team Members



**SOPHIE**

Manages the MPS Advocacy Team



**STEVE**

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



**ALISON**

Supports members living in Ireland



**DEBBIE**

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



**REBECCA**

Fabry  
MPSII Hunter  
MLIII / MLIV  
Mannosidosis, Fucosidosis

### New Palliative Care & End of Life Booklet

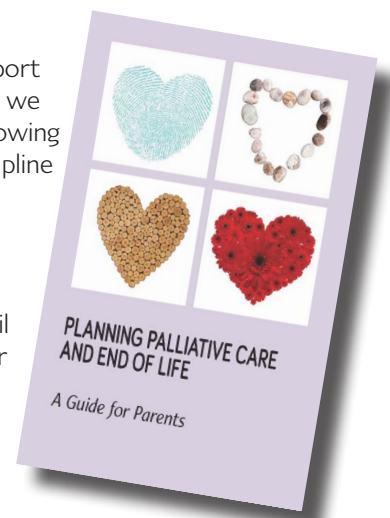
The MPS Society has produced and published a booklet entitled *Planning Palliative Care and End of Life - a Guide for Parents*, which sensitively covers both the approach to end of life care, and the choices available to you, as well as grief in the aftermath of a death.

We have ensured that chapters are marked clearly so that readers can choose what they feel ready to approach and we have also included contact details of organisations who will be able to offer a range of helpful services.

Of course our Advocacy Support Officers are also able to provide a supportive role in the planning of palliative and

end of life care. We provide support at the time of bereavement and we are still here to support you following your bereavement offering a helpline service and practical advice.

If you would like a copy of *Planning Palliative Care and End of Life - a Parents Guide*, please email [advocacy@mpsociety.org.uk](mailto:advocacy@mpsociety.org.uk) or call 0345 389 9901.



### Bereavements:

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

**Pavan Tailor** who suffered from Multiple Sulphatase Deficiency and passed away on 30th November 2015 aged 19 years.

**Marjorie Banks** who suffered from Fabry disease and passed away on 11th October 2015 aged 67 years.

**Hayleigh Reynolds** who suffered from MPSI Hurler Scheie disease and passed away on 29th November 2015 aged 17 years.



# Clinics

Royal Manchester Children's Hospital MPSI BMT clinic - 16th October 2015



## Clinics at the Royal Manchester Children's Hospital by Pauline Hensman

I have worked for over 20 years at the Royal Manchester Children's Hospital. My role is part of the metabolic team as the children's physiotherapist for the Lysosomal storage disorders, mainly children with MPS disorders. I have had the privilege of knowing the children and their families from when they are first referred as babies/toddlers, through the years until they reach transition and eventually leave us to join the adult service. I may see the children and their families in many locations depending on their need at the time, this may be in the therapy department for assessment, in various out-patient clinics, on the wards after undergoing surgery or as part of research trials.

Getting to know the children as they grow up and develop can be so beneficial when they need to see 'new' doctors or if the need for surgery arises. Along the way good communication with the local therapists and school teams is a vital part of my role. The sharing of relevant information can assist with better planning for appropriate equipment and any potential adaptations. Education, presenting talks at various meetings to health professionals, schools and to the families directly can be a very useful way to pass on practical information that can help on a day to day basis.

If you ask the children what I do, they'll say 'blowing' games (lung function tests), play on the mats, lots of walking (6minute walk test), and of course stickers - yes, you're right.....we do in fact have a great time!

*Pauline Hensman  
Highly Specialised Physiotherapist in Lysosomal Storage Disorders Therapy and Dietetics Services*



Melissa & mum



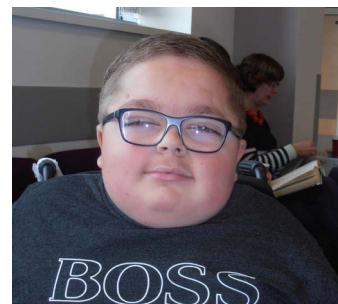
Rachel



Alicia



Cody



Thomas



Birmingham Children's Hospital Fabry clinic - 23rd October 2015



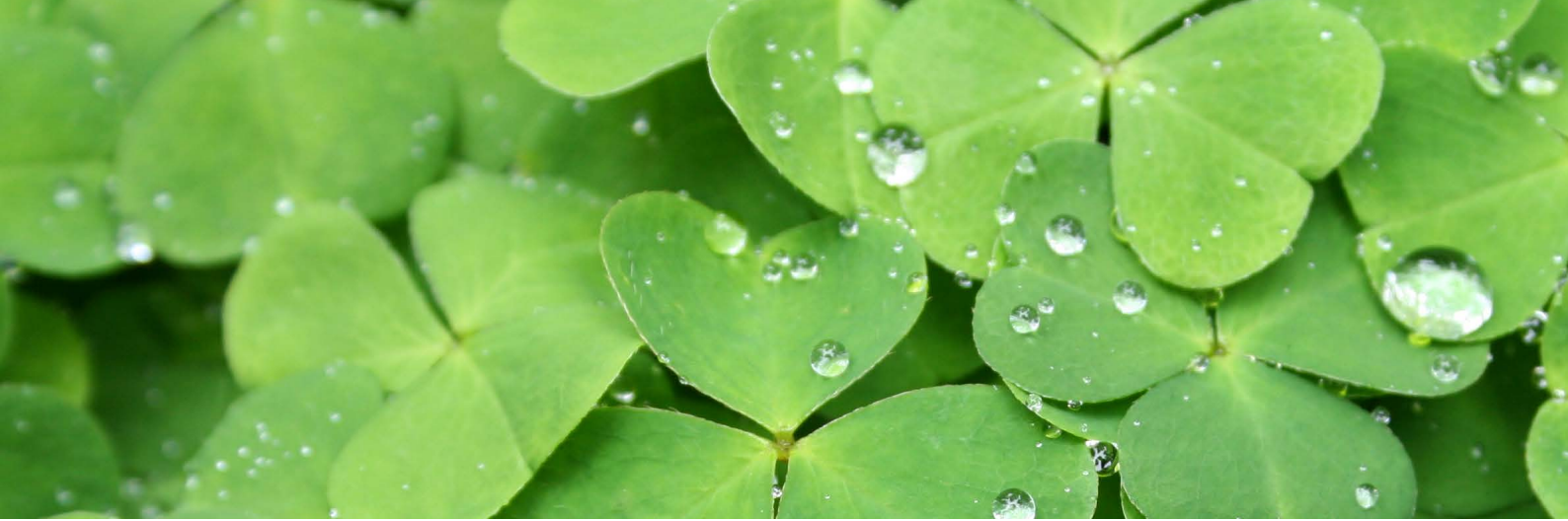
Birmingham Children's Hospital MPS clinic - 27th November 2015



GOSH MPSIII clinic - 8th December 2015







## All Ireland Advocacy Support Update

It's hard to believe that we are already into a new year and that 2015 is almost a dim and distant memory! By the time you are reading this you will have had your Christmas break and will probably be back into your usual routine. Sometimes the New Year can be a bitter sweet occasion; while wishing each of you a happy and healthy 2016 I want to remind you that if this year brings you challenges and difficulties, the MPS Society are always there to stand by you and help you through.

New Year's often bring about change, and 2016 is bringing some changes to how we deliver clinics in Northern Ireland. As we expand and develop our local MPS clinics you can expect a few changes in order to ensure that each of our members have enough time and space at our specialist clinics to address all their needs. Our clinics will remain in their usual locations and I will be in touch with information about the clinic schedule early in 2016. If you have any questions in the meantime, or if you feel you need to be seen at clinic sooner rather than later, please do not hesitate to get in touch.

As usual we've had a busy time over in Ireland over the last season. I've travelled both North and South supporting families through both new and difficult experiences and as always it has been both inspiring and humbling to watch you all navigate new territory and overcome difficulties. 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](mailto:a.wilson@mpssociety.org.uk)



### The Sights and Sounds of Christmas

Our Annual Christmas party this year was a great success. We were delighted to welcome a range of families - from our very longest standing member to our newest family - to a magical night of festive music, fairy lights and lots of Christmas crafts. The Children enjoyed making some lovely gingerbread creations, decorations and Christmas cards. While the adults reminisced and caught up with each other over cups of tea and coffee.

These get-togethers are always a wonderful opportunity to meet friends, old and new, and to let your hair down and have some fun. Our thanks to the Enkalon Foundation for kindly funding this event.

*Alison Wilson  
Advocacy Support Officer*

### Save the date - All Ireland Conference, 20th - 22nd May 2016

2016 is the year of our All Ireland Conference in The Hilton Hotel in Templepatrick. The conference programme is prepared and we know that this year will be a excellent one. We have lots of new families who have never been to one of our conferences before and we would be delighted to welcome you to this fantastic event.

While the adults learn more about MPS and its management the children always have a fantastic time as they team up with trained volunteers and enjoy our childcare programme.

If you have any questions or would like to find out more please give Alison a call.





## Your Stories



*Chiara's First Day at School*

Chiara Fox is 5 and is 3 and half years post BMT (bone marrow transplant). She was diagnosed at 8 months with MPSI, Hurlers. She commenced ERT (enzyme replacement therapy) as soon as her diagnosis was confirmed and had her 1st BMT in August 2011 but rejected in December 2011. Her 2nd BMT was in May 2012 with her donor being her dad.

Chiara started P1 at Moray Primary in Grangemouth in August 2015. Chiara currently is loving school and goes between an enhanced provision class and mainstream. She manages full day no problem and is very sociable. On top of starting school she has joined an evening Rainbows group which allows her to take part in activities like every other girl her age. Between starting school and Rainbows we could not be more proud. It was a day we never thought we would get. It hasn't been an easy journey as Chiara has also had grommets inserted, tonsils and adenoids removed, spinal surgery with fusion, bilateral hip reconstruction, and two lots of BMT. All of it taken in her stride.

*Michelle Petersen*  
*Chiara's mum*





## The Lions Back Harvey Brown!

*Young Lions fan, Harvey Brown (MPSIVA) was supported in his campaign to get Vimizim funded by Millwall Football Club and his schoolmates from Surrey Square School. The Brown's wrote in with details of Harvey's adventures!*

Millwall have been absolutely brilliant in what they have done and are doing for Harvey. They maybe a small club but they have one big heart.

Harvey was invited to the training ground to meet the players and staff - the way he was treated by the manager and players was unbelievable. He is even on first name terms with them all. We were then invited for a V.I.P day at the Den for the game against Chesterfield. He was invited into the changing room before the game, then pitchside to watch the players run onto the pitch, then taken to watch the game from the executive seats.

Soon after he was chosen to be a mascot at the Rochdale game; he was invited onto the pitch to kick the ball with the players, and he even got to choose a player to walk out of the tunnel with (he chose Aiden O'Brien). He turned out to be a lucky mascot as we won 3-1! We were escorted to our seats and as we walked over the fans gave Harvey a standing ovation and were shouting his name, which was so

emotional. I could not hold back the tears, but Harvey took it all in his stride and waved to the crowd.

The club has really researched Morquio and put all about the condition and his petition in the match day programmes, on their main website and also have been tweeting about Harvey and Morquio. Also last Friday 23rd October Millwall took us along with some pupils from Year 6 to deliver their hand written letters to David Cameron at 10 Downing Street on the Millwall bus, along with the press team and Club mascots Bolina & Zampa, then on for a little tour of London then back to school.

The photos above are from the last few months of Harvey's adventure. We really did not realise that when Richard White at Millwall got in touch with us after reading about Harvey in our local paper the Southwark News, that it would have got this much coverage and help.

*Vikki, Dean & Harvey Brown*



*Our thanks to Millwall Football Club and Surrey Square Primary School for their incredible support for Harvey Brown and the Vimizim campaign!*

*We've seen some amazing press coverage - everything from the Southwark News to an interview with Harvey and his family on ITV London!*







## Jibseekers: Living with Morquio

*photo courtesy of Inglenook Productions*

First thing you need to know: I'm weird. Hold onto that thought. I wake up every morning, at whatever time I need to be up, and check for pain. Ankles, knees, wrists, and back. Seriously, last week I woke up with a bad ankle and spent the following three days hopping around. What 26-year-old does that? Decides a broken leg is something you walk off and gets on with life? Definitely not a sane one - maybe a rare one.

Some say there's something wrong with me; an issue, or a concern. Those in the know call it MPSIV, Morquio. I call it a thing. Society says I suffer from Morquio, but I disagree, I live with Morquio. It is a part of me, like my legs or personality. The thing about genetics, especially mutations, is the odds of getting them. Essentially, I've got two dodgy genes, which mean I can't make a certain enzyme, which means I can't break down a certain protein. The odds are 1 in 250,000. If I'd put money on those odds I'd be a rich man.

What does my regular day involve? Get dressed and breakfast, usually toast, then off to work. I work in an office, 9 to 5, 4 days a week. It's strange the things you notice when you really look. Saw a cat the other day, found myself thinking why, why did a cat become a pet? What possessed the king of the jungle to attach himself to what is essentially a clumsy ape? Questions without answers. It's good to think, and to question. How did yesterday pass by? I have spent the last 26 years living in the moment, focusing on the positives, leaving the past behind me. My mindset is to forget a day once it's over, it drove my parents insane during exam time, and still does. But I invite any of you to come with me, to

take a trip down the rabbit hole.

The four-day working week is significant, as the fifth day is given to science. It's been three years now since I started moonlighting as a guinea pig, all because of that 'thing'. New ideas sound obvious once you really start to think about them, yet it takes a genius to ask the question. He's missing an enzyme, so what would happen if we gave him that enzyme? There really is a fine line between genius and insanity, like the bloke who discovered you could drink cow's milk. Call me sexist but it must've been a bloke, most women I know wouldn't look at a cow and think "I'm gonna pull on that dangly thing and drink whatever comes out". Anyway, that's how the treatment, Vimizim, came to be licensed. All because someone wondered, "what if?" and I'm pretty glad that they did. Vimizim is now funded in many countries around the world, and currently the UK isn't one of them\*. I've waited a long time for a decision to be made, which for someone with a life-shortening condition is a bit harsh, but I'm crossing my fingers that things are about to change.

I'll let you in on a secret: people don't talk to me. Maybe because they're scared of offending, but what are they scared of? I'm three feet tall, what's the worst that could happen? The worst blokes could expect is a head butt to the balls. People say that all you need to do to succeed in dating is make a woman laugh. My problem is getting the conversation started, because so far, the women I've met just get maternal. Not the result I'm looking for! Take the chance, risk offending me, get to know me, and you might like the guy who answers back.



The fun starts after work, once the sun goes down. Buying a pint of beer is always tricky when you're my height, but you just need to be ready when they drop it off the bar. I like to go to a gig, watch a bit of stand-up, and perform a bit of stand-up too. Live comedy, an art form that can't be censored. I've always had a sense of humour, and enjoyed giving presentations, so stand-up seemed like the next natural step. It's a world I wouldn't have known about if I hadn't taken a chance at university. The e-mail said "Do you have a sense of humour? Join the comedy society if you do." I figured it seemed like a laugh. That first ever gig I died on my arse (comedy at lunchtime before the union meeting wasn't the greatest idea) but the buzz, man, it was intense. From that moment I wanted to go again, because you can't beat the feeling of standing on a stage. I've been performing for about four years, mainly talking about my life. It's strange how you can feel so safe when standing, stripped bare, on a stage. My friends say that when I'm up there, the disability disappears.

My weekends are for relaxing, and I enjoy travelling around the UK

and abroad whenever I feel able to. I've visited a few places abroad and Amsterdam has to be one of my favourites. There's very open-minded people there and a lot of bikes, and I absolutely love cycling. I've got short legs but when I'm on my bike I'm just as fast, if not faster than everyone else. I cycle on the roads, with traffic; nothing like a bit of danger to get the adrenaline flowing. Whichever way you look at it, it's the closest feeling I'll ever experience to flying. I've been told it's risky, a vulnerable position to put myself in. Anyway, you can't hit what you can't see. There is something magical about riding a bike, and I may not be the next Bradley Wiggins but I love knowing that the humble bicycle is the only vehicle where its passenger is also its engine.

So what does Vimizim actually do? It ain't a cure, more like a supplement. There isn't a cure, no secret potion or magic wand. There's a typo in the genes, the instructions are wrong, and the factory has stopped making the part I need. Vimizim is that part, it clears the rubbish out of my

***"Take the chance, risk offending me, get to know me, and you might like the guy who answers back"***

system. Imagine living in a house where no one empties the bins, a student house for instance. The theory goes that Vimizim lets everything run at its maximum efficiency, and in practice? My breathing is easier, my eyes clearer and I have so much more energy. The average person might live their life with an angel on one shoulder and the devil on the other. So far I've had to deal with a third guy, let's call him Body. When I used to cycle, I would ask for an extra 10% but Body would say no, make me stop, get off and push or even wait and rest. Now, with the help of Vimizim, when I ask for an extra 10%, Body says to me "have 20%". When I'm on the treatment, I know exactly what my limits are. There's no second-guessing, and no half measures. It means I can climb higher, move faster, become stronger.

This is my life as I know it. It's easy, tough, fun and hard work all at the same time. How long will it last? I don't know. The treatment I need is costly, and I'm a rare species. In a time of cutbacks and pressure on the NHS, it's hard to digest the facts and figures. All I know is that when you add up the cost of a lifetime's

supply of appointments, clinics, operations, adapted bikes, cars, wheelchairs, medication, therapies and job seekers allowance, Vimizim seems a good value alternative. Some of the kids out there who have been on it from an early age are already a good few inches taller than me, so that's saying something.

There's a whole world out there, just waiting to be explored, and I fully intend to explore it. After all, why should Higher, Faster, Stronger only apply to Olympians? Here's to Vimizim, and may those in charge see sense and keep getting the bins emptied. And if they don't, then I'll just have to move to Amsterdam.

*Jibreel Arshad*

*\* NOTE: Article written before the announcement that NHS England would be funding Vimizim.*

## *Crowdfunding helped support early success in Vimizim campaign*

A big 'thank you' to everyone who supported our Crowdfunder appeal to raise funds for Vimizim and to support children and young adults with Morquio disease.

We launched the appeal on 3 July and it raised £6,842. In total 164 pledges were made by the end of July when the appeal closed. This money has been used to support the Vimizim campaign which really made the difference in achieving a 'Yes' from NICE and NHS England. The unprecedented publicity, in Parliament and within the media, has raised awareness and has really changed minds. On 2 September NICE announced that it would "provisionally recommend Elosulfase alfa [Vimizim]... if specific conditions are met. On 16 December NICE approved Elosulfase Alfa for use through the Managed Access Agreement in England "

Speaking on behalf of the MPS Society's Trustees, Sue Peach, Chairman, said "The challenge of securing access to Vimizim for Morquio patients has been huge. Keeping up the fight has been very demanding on the MPS Society as we are only a relatively small charity. We are really grateful for the support that the campaign has received and the Crowdfunder initiative certainly helped us keep up the pressure on Governments in England and Scotland. Right now this continues to be particularly important for families in Wales and Northern Ireland."

The Crowdfunder initiative helped send a vital message that the NHS was operating a discriminatory policy by denying treatment to children born with ultra-rare diseases. Sue added that the outcome of the campaign was vital as it has implications for how other potential new treatments for MPS and related diseases may be viewed in future.

# Events

## Merlin's Magic Wand Chessington/Alton Towers Family Days

Merlin's Magic Wand is a worldwide charity for children and they arrange magical days out at Merlin Entertainments attractions. They welcome applications on behalf of children aged 2-18 with a confirmed serious illness/long term illness, disability or disadvantage, who would benefit from a day out at a Merlin attraction.

In September our application was successful and The MPS Society was sent tickets from Merlin's Magic Wand to their Chessington and Alton Towers attractions. We were however, only allocated a small amount of tickets so we offered these on a first come first serve basis, and they were snapped up in no time at all!

The feedback from the families was fantastic and it sounds like a great time was had by all that attended. If you missed out this time then make sure you keep an eye out as we will be applying for more tickets early next year!

*Emma Henry  
Trusts & Grant Fundraising Officer*



### *Lois & Clark's Day at Chessington*



We used our Merlin's Magic Wand tickets and had a great day at Chessington. It's local to us and the kids have been with their schools a few years ago, but we haven't been as a family since they were both small (Clark is now 8 and his sister Lois is 13). The weather was dry and not too cold and it wasn't too busy for a weekend. Clark enjoyed going on lots of rides, including Bubbleworks, Tiny Truckers, Sea Dragons, Flying Jumbos, Toadie's Crazy Cars, Zufari, watching the Madagascar show and seeing the sealion, penguin shows and animals. Clark loved the Octonauts and the fish in the SeaLife centre.

*Cath D'Alton  
Mum of Clark D'Alton - MPSII Hunter*

## *A day out at Chessington World of Adventures*

– “Daddy, daddy, daddy! Are we there yet?” – Benji asked.

– “We have just left Canterbury, Benji. It will be a long journey. Look boys! There are hundreds of sheep in that field on the left side. I said left. There! Okay, never mind I am sure we will see some more later.” – Mummy answered.

– “What is our destination today?” – Benji asked again (he learnt this word from the “lady who speaks inside the SatNav”).

– “The destination today is a big-big amusement park, called Chessington World of Adventures.” – Daddy said. “There will be lots and lots of amazing rides, like Scorpion Express, Bubbleworks, Safari Skyway and there will be wild animals and sharks and many other lovely creatures.”

– “Will there be Tigger as well Mummy?” – Gabriel asked.

– “Of course Sweetie, the real one.” – said Mummy and Daddy together.

After arriving we could go straight in without waiting in the long queues because we already had the pre-booked tickets. The first ride was in the Bubbleworks which was great fun for all of us. Then we had a ride in super-fast sailing boats called Seastorm. The boys were laughing and yelling continuously during this ride. Hungry boys got their lunch, then came the Carousel with Mummy which was followed by the Tiny Trickers. Both Gabriel and Benjamin just could not wait for this ride to come and were a bit disappointed when it came to an end. Fortunately the Sealion Show has just started and they forgot the trucks almost immediately.

Then we were walking around to see the “Tiggers”, lions, binturong, gorillas, otters (they were wrestling in the water so cutely), snakes, arthropods and the Sealife of course. The young adventurers had then a great play above us at Amazu playground and got their well-deserved freshly made doughnuts. Lastly we all jumped in the Scorpion Express



which was maybe the best ride to close the day.

At the gift shop we went in there were mostly plush animals like those in the park. We have given the “order” that each boy can pick up one toy only.

– “So, what did you choose Gabriel? A Lego excavator? Come on! There are lots of other soft toys like the animals we saw here,” we said.

– “No! I want this excavator. Please.”

– “Look Gabriel what Benji have chosen! It is a nice plush monkey! Oh, okay. Benji chose a Lego racing car instead.”

To be honest we are so proud of them because these 3- and 5-year-old children were able to choose the one and only creative toys for their age in that shop instead of having the fifth “Tigger” or the twelfth Teddy bear in the corners of their beds.

On the way back home there was silence in the car. No, no one was sleeping and no one was watching the landscape. Benji and Gabriel just had new adventures on their tablet computers.

This was a whole day’s fun with lots of good experiences for all of us.

Many, many thanks to The MPS Society and to Merlin’s Magic Wand for providing the tickets and the adventure for us, especially for Gabriel and Benjamin. The boys are still mentioning the rides and the animals and want to go back – and they also love to see the pictures taken there again and again.

Thank you.

*Benjamin and Gabriel (MPS II both) and their parents*







## Manchester & Liverpool Pantos

Sophie and I started out at 6.30am on Saturday morning to make our way to Manchester. In true style the train was a little delayed but we made it to the Copthorne Hotel, who do such a brilliant job of looking after us and our families. On arrival we set about dismantling the tables and moving the cutlery, ready just in time as the pantomime team arrived to set up. We sat down to a delicious hot buffet to begin, and once we had finished the panto Jack and the Beanstalk began. I have to say I am not usually enthralled by pantos, but this was fantastic and gripped the audience for the whole two and quarter hours. The crew of five were amazing, even if they did have to add in a few extra lines because some of the younger members of the audience had climbed on to the stage area looking for the cow, which happened to be the star of the show for the children!

By this time the wind was howling and the rain was pouring and we said good bye to all the families.

We then ventured out in the rain to Liverpool. We arrived in Liverpool to even faster winds and heavier rain, but we struggled through the downpour and finally made it to the hotel, which was a story in itself. Sophie and I managed to get the last room, complete with a broken shower and a double bed. To cheer ourselves up we decided to have a snack, only to be told that they were out of what we both had ordered! With that, we decided that it was time for bed!

We arrived at the Atlantic Towers hotel around 11am, just as the panto team were setting up for their performance of Beauty and the Beast. Again, a brilliantly entertaining performance by a great team of actors, followed by a wonderful meal. With all the excitement over and done with, and lots of happy memories made, we wished everyone a safe journey home.

*Gina Smith  
Group Finance Officer*





## Gulliver's Land Festive Family Day

On 22nd December 2015, a group of families and a few MPS staff members visited Gulliver's Land Theme Park in Milton Keynes to enjoy a festive day of fun in the lead-up to Christmas.

Gulliver's had created a Winter Wonderland of rides and attractions to keep the whole family entertained. Of course, the day would not have been complete without a visit to see Father Christmas in his grotto, who presented all the children with a well-deserved gift!

Not leaving all the hard work to her husband, Mrs Christmas was on hand to show everyone around her kitchen to cook up some tasty treats, plus there was the opportunity to become an honorary elf by attending Elf School.

A festive feast was also on the menu, with all the trimmings - just to get everyone in the spirit of the season before the big day arrives!

Our thanks to BBC Children in Need for so kindly funding this family day.







## 14th International Symposium on MPS & Related Diseases

*14th - 17th July 2016, Maritim Hotel, Bonn, Germany*

The 14th International Symposium on MPS and Related Diseases will take place in the Maritim Hotel Bonn, Germany, from 14th – 17th July 2016.

During the four day conference, delegates will learn about the latest developments in research in the field of MPS and related lysosomal storage diseases.

As in previous years, the international symposium in 2016 will be a great forum for discovering what is new in the field of metabolic diseases research, which includes:

- Researchers, doctors, physiotherapists etc. discussing the latest information on MPS and related lysosomal storage diseases
- The latest in international research
- Special workshops for patients / family members
- Plenary session for all participants, scientific and patient program
- A unique opportunity to meet the researchers, doctors and clinical staff members leading the field in metabolic diseases research and the chance to share your experience with peers from all over the world
- A social program for accompanying persons
- An exciting program for children and young people





## *Would you like to attend?*

With the 14th International MPS Symposium taking place in Bonn, Germany in a little over 6 months from now the MPS Society is offering its UK members / member families the unique opportunity to participate in this pioneering meeting. The Symposium starts in the afternoon of Thursday 14 July and finishes at lunchtime on Sunday 17 July. As you will see from the enclosed programme there is something for everyone; state of the art clinical management, advanced nursing care; research and breaking news and clinical trial development with input from many of the pharmaceutical and biotech companies with therapies and pipeline drugs.

If you are interested in being supported to participate in this important meeting please complete the enclosed form and return to MPS House no later than 30 January 2016. Places are limited so please get your application in early. Please be aware that submitting an application does not guarantee your place/s. **Only book directly on the Symposium website [www.mps2016.com](http://www.mps2016.com) if you are paying yourself and not wanting to be part of the UK MPS Society contingency.**

### **What the UK MPS Society is offering its members:**

Places for up to 5 MPS adults, each accompanied by a companion / carer

Places for up to 10 MPS families with an affected child / young adult (up to 2 adults and 2 children)

The MPS Society will pay the conference registrations and childcare registrations; 4 nights in the Maritim Hotel, Bonn from Wednesday 13 July – Sunday 17 July 2016 and provide a travel bursary that can be used towards flights or to travel by train or car.

Additional adult /child price on request





## Childhood Wood Planting 2015

Each year the MPS Society hold a special day at Sherwood Pines Forest Park for our bereaved families. Families are given the opportunity to plant an oak tree in memory of their loved one at Childhood Wood, a site dedicated to those who have lost their lives to MPS and related diseases. This year the event was held on the 25th October. We met at the Clumber Park Hotel to be welcomed by Christine Lavery (CEO of The MPS Society), Wilma Robins (MPS Society Trustee), Councillor Fielding (Nottinghamshire County Council), Consort Ray Fielding and Commander Helen Swann for lunch before heading to Sherwood Forest.

At the Forest we gathered in the car park, and headed down the white trail to Childhood Wood, with the clouds clearing and the sun shining. Here we heard about the importance of the wood, what it means to the local community and for those all across the UK. Wilma Robins then read the poignant poem "Remember" by Christina Rossetti.

Those being remembered were:

Faye Barnet, Reece Culverwell, Jade Lauren McAfee, Nicolina Stangoni, Joseph Tilling, Richard Lewis Turner, Natasha Pace, Sam Hitchin, Daniel Lewis Muers, Max Alan Cawkwell, Simon Mansfield, Andrew Millar.

Following the reading family members released balloons, with personal messages attached, into the sky before taking an oak sapling and planting it in the prepared area.

I would like to take this opportunity to thank Commander Judith Swann, Councillor Fielding, Consort Ray Fielding and Wilma Robins for taking the time to be part of our event. Also thanks to the forest rangers for their hard work and support.

*Steve Cotterell  
Advocacy Support Officer*



## *International Conference: Sanfilippo Syndrome and Related Lysosomal Storage Diseases. 26th - 28th November 2016 Geneva, Switzerland*

Last month, we flew to Geneva to attend the 2nd Sanfilippo Conference hosted by Alliance Sanfilippo. The conference ran for three days. On the first day, there were a few presentations followed by a welcome drinks evening, where we met other parents from across the world, including another parent from the UK.

The following morning, it started to snow, which we were very excited about as we don't get much snow at home in Dorset!

The first day of the conference was an early start - 8.30am - and was filled with many talks by leading experts in Sanfilippo and other lysosomal storage diseases. These fascinating talks included genetics, orphan drug development, natural history of Sanfilippo and updates on clinical trials, including enzyme replacement therapy and gene therapy, some of which are ongoing and others to start in the near future, including gene therapy. Dr Brian Bigger spoke about developments towards a stem cell gene therapy treatment for Sanfilippo A, research for gene therapy for Sanfilippo C and an update on the Genistein trial ongoing in Manchester.

There was a fascinating talk by Dr Frits Wijberg on the outcomes of various studies of natural history of patients with Sanfilippo syndrome and the huge variability of the disease in different children. There were cases of children in Holland who were extremely attenuated, meaning that the progress of the disease was much slower. Among these cases, he spoke about two boys, one who was shown by video waterskiing and a picture of another boy who had recently taken his driving test! We did giggle at this, as we couldn't imagine our own son, Daniel driving a dodgems car safely on his own, let alone a real car!!

At lunchtime, we were really excited to meet Daniel Schuepbach. Daniel, who is nearly 25, lives in Switzerland and has MPSIIIA. I had been in touch with his mother, Elizabeth, soon after our own son, Daniel was diagnosed, and we met her in Switzerland a few years ago. Elizabeth sadly died last year. Daniel is a very handsome young man who came to the conference with his father and uncle, and was looking really well. He attended one of the lectures, sitting quietly at the table, eating crispbread!

On the final day, there was a round up of topics which had been covered in the conference and a chance for families to ask questions.

The conclusions were that research and possible treatments for Sanfilippo have come a long way in the last ten years. At present, there are a number of trials ongoing in Europe for MPSIIIA and B, with some due to start in 2016, including:

- Intravenous enzyme therapy
- Intrathecal enzyme therapy
- Intravenous gene therapy
- Intracerebral gene therapy
- Autologous stemcell gene therapy
- Chaperone therapy
- Substrate reduction therapy

There are also treatments for MPSIIIC and D in the pipeline. We would like to thank Sanfilippo Foundation Switzerland, Corinne Fery-von Arx and Frederic and Stephanie Morel for an amazing conference, wonderful hospitality and making us feel so welcome.

*Rachel and Edgard Zaldua*



# Research & Treatment

## Research on Oral Nutritional Supplements

The Society has been contacted by Liberating Research, who are conducting a piece of research looking at the use of Oral Nutritional Supplements (pre-made pouches only).

The purpose of the study is to find out how paediatric patients use Oral Nutritional Supplements and what their experiences are with these products. From the input and feedback received they hope to be able to identify unmet needs and areas for improvement.

The company would like to emphasize that this is a market research study with no promotional purpose. All of your responses will remain anonymous and only be analyzed and reported in an aggregated format together with all other respondents.

### Format of the research:

Interviews will be in the format of a face to face interview in the home and will take approximately 60 minutes. Ideally they would like to interview both parent and child (the child ideally needs to be between the ages of 6-12) as we would also like to ask a few questions to them as well – how do they like the taste, how easy is it to use etc alongside questions to the parent. Obviously this is only relevant if the child is well enough to contribute. They will try and make the research quite interactive, so the child can draw pictures, respond to images etc. £80 will be gifted to the family for their time.

The basic criteria for the research is as followed; just have to be a family using Oral Nutritional Supplements 3-4 times a week, have been prescribed the ONS by a physician and the child is between 6-12 and all are happy to take part.

If you are interested in finding out more or would like to take part in this research, please contact Carly Brown (Co-founder & CEO of Liberating Research) on [carly@liberatingresearch.com](mailto:carly@liberatingresearch.com) / 0207 249 4461

*\*This study is not endorsed by the MPS Society and any agreements to participate / share information is strictly between the patient and Liberating Research*

## MPSII Treatment Update

Treating the severe form of MPSII remains a clinical challenge due to the limited availability of enzyme to the central nervous system. Various treatment strategies are currently being developed in the hopes of providing more enzyme through the blood-brain barrier and having a long-lasting impact on the brain.

ArmaGen Technologies has recently designed a modified version of enzyme replacement therapy by fusing an antibody to the human IDS enzyme (AGT-182). This fusion enzyme acts as a molecular Trojan horse and aims to deliver high enzyme activity to the brain to reduce storage within brain cells. AGT-182 is currently being tested in a phase I clinical trial for male patients aged 18 years or older (NCT02262338).

Intrathecal delivery of replacement enzyme is also currently underway in various centres worldwide. Shire has revised its formulation of intravenous idursulfase, which does not cross into the brain, to produce idursulfase-IT, a suitable formulation for delivery into the cerebrospinal fluid. Intrathecal injections bypass the blood-brain barrier by directly delivering small volumes of replacement enzyme using an IDDD (intrathecal drug delivery device). Injections of 10mg of idursulfase-IT are given monthly for 12 months. This study is currently recruiting participants up to 18 years of age (NCT02055118).

Direct AAV-mediated gene transfer to the central nervous system is also being investigated at the pre-clinical stage but no data has so far been published.

Our approach, haematopoietic stem cell gene therapy (HSCT-GT), modifies the patient's own bone marrow cells to replace the entire blood and immune systems. This strategy also allows corrected cells to cross into the brain and become 'enzyme reservoirs' for surrounding brain cells. Bone marrow cells are modified by a lentiviral vector designed to re-introduce a correct copy of the IDS gene, and would be given as a one-time treatment. HSCT-GT is being tested in the MPSII mouse model after successful in vitro testing and should result in correction of neurological symptoms.

*Hélène Gleitz  
Stem Cell & Neurotherapies,  
Manchester Centre for Genomic Medicine*

## New National Homecare Specification for LSD Services

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'As part of the national specialised service for lysosomal storage disorders, patients with an LSD are able to receive care that allows them to receive their fortnightly infusion in their own home. The first ever national framework (covering all eight LSD centres in England) ran from 2012 until it finished at the end of September 2015.

Representatives from each of the eight LSD centres, with representation from the UK LSD Patient Collaborative, have been working hard for the last 18 months to update the specification. Taking learnings and feedback from the last specification, the new framework began on the 1st October 2015. We are delighted to announce that a third company, Pharmaxo, will join existing providers Bupa and Healthcare at Home on the new framework, which will offer more choice to patients.

Patients and family members will hopefully see no major changes with the new homecare framework and we are hoping for a seamless transition and service as normal', writes Tanya Collin-Histed, Chief Executive of the UK Gauchers Association.

Alison Davies, General Manager at Pharmaxo introduces the company: 'Based in Corsham in Wiltshire, Pharmaxo has been offering innovative, high quality pharmacy services since

2010. Building upon this success we commenced homecare services in October 2013 providing home infusions. We have continued to develop our areas of expertise and provide homecare across a range of therapies including oncology, immunology and respiratory conditions, and now, LSD.

Pharmaxo, drawing on the experience of our sister organisation Bath ASU, develops services which are tailored to specific therapies. This enables us to focus on meeting the needs of the individual whilst maintaining quality and choice for all patients. By concentrating our attention on specific therapy areas we feel that we can invest in developing innovation in homecare which can deliver enhanced services and better value for patients and commissions. Nursing services are delivered through our nurse provider, Ashfield Healthcare.

With 15 years' experience developing, designing, building and delivering clinical support programs they have the capacity and capability to develop LSD services with Pharmaxo, the NHS, patients and their families.

We are delighted to have been successful on the LSD Framework and are looking forward to working with the specialist centres in providing consistent high quality homecare services to patients and their families.



# Information & Resources



## Right Chair, Right Time, Right Now

The Wheelchair Leadership Alliance was formed in January 2015 under the leadership of Paralympic athlete, Baroness Tanni Grey-Thompson.

This Alliance is a commitment group campaigning for a better deal for wheelchair users, and aims to transform the quality and effectiveness of services across England. Wheelchair Leadership Alliance is running a national campaign to raise awareness of issues in wheelchair services and show how people's lives can be transformed with the right chair. The campaign is targeting two key audiences, firstly the commissioners, providers and manufacturers who can make changes. Secondly, those who can demand changes: wheelchair users and carers.

The campaign is underpinned by the Wheelchair Charter, which outlines the crucial principles for the commissioning and delivery of high quality wheelchair services that will help people live their lives to the fullest potential. To pledge your support for the Right Chair, Right Time, Right Now campaign, or to find out how you can contribute to the discussions go to [www.rightwheelchair.org.uk](http://www.rightwheelchair.org.uk)

## Beach Wheelchair

There is currently a small group campaigning for beach wheelchairs to be available for hire on Blyth South beach in Northumberland. Other schemes on Great Yarmouth, North Berwick, Brighton and Hove and at least ten Cornish beaches are really popular.

In September, people were welcomed to Blyth beach to test out a selection of wheelchairs from local manufacturer, Adventure Mobility. The day was very successful and for some it was the first time they had ever been able to be on a beach. The organisers were worried at one point that the chairs wouldn't be returned as families had so much fun!

For more information on the campaign and existing schemes go to [beachwheelchairsforblythsouthbeach.wordpress.com](http://beachwheelchairsforblythsouthbeach.wordpress.com)

## MPS Sibling Book

If you are the sibling of an MPS sufferer, you may be interested to learn that there is a book available which includes 28 stories from MPS siblings and caretakers from around the world. These stories demonstrate how siblings cope with situations you may be experiencing at home. Have you ever felt angry, disappointed, sad, guilty, or jealous? Do you get upset when people stare at your sibling? If so, read this book as you embark on your journey with your MPS sibling.

*The MPS Sibling: Short Stories for Brothers and Sisters* by Nathan Grant is available from [www.amazon.co.uk](http://www.amazon.co.uk).

## New Help for Wales

New guides to help adults with a learning disability and their carers access health and social care services have been launched by Mencap Cymru in conjunction with Cardiff University's School of Law.

The toolkits will provide a one-stop shop for information relating to health and social care services, including the duty and obligations of local authorities and health boards and the legal framework governing the challenges of decisions taken by these bodies.

For more information visit [tinyurl.com/dhgwales](http://tinyurl.com/dhgwales)

## Assist-Mi App

Having a disability sometimes means that accessing services and planning journeys can be that much more stressful. Assist-Mi is a free app designed to empower its users, enabling greater access to services.

As their website explains:

*“service providers can anticipate an individual's arrival and requests for assistance and purchasing goods and services can all be made, acknowledged and actioned in real-time.*

*Additionally, the service provider will understand the accessibility needs from user's personalised Mi-Profile, so the disabled user can arrive with full confidence that their individual requirements will be met.”*

Visit <http://www.assist-mi.com/> for more information.



Elliot Moody took on a 50 mile non-stop race across the Lake District and raised £2,820 for the MPS Society.

Read more about Elliot's incredible Ultra-Challenge on pages 32 - 33

## Fundraising

Thank you to everyone who supported us during the festive season by buying and selling raffle tickets and purchasing our MPS Christmas cards - it was a wonderful end to a fantastic year of fundraising.

This edition of the magazine has some inspirational stories from our members and supporters, who took on a range of diverse challenges, from head shaves to skydives. It really does show how that fundraising is very much a personal choice, and you really can make it what you want.

Speaking of challenges, we have secured our charity places for various events in 2016, including the British London 10K and the Great Manchester Cycle - take a look at page 39 for a full list of our places. Don't forget that you are able to enter these types of events in your own right and still fundraise for the MPS Society, so if you like the look of a

particular event, go for it!

If you are planning any fundraiser, do remember to let us know what you are doing and when. We can offer a great fundraising pack to help you on your way, which contains posters, leaflets, a useful guide and balloons. Our website also offers a wealth of information on fundraising, so it may be worth taking a look if you are stuck for ideas.

In addition to our website, our Facebook and Twitter pages also contain lots of useful news and fundraising ideas, so remember to like/follow us. Feel free to post to our pages to let everyone know about your fundraiser. We love to see your photos too!

*Elkie Riches*  
Fundraising & Information Officer



## Thank you Highcrest Academy!



Earlier on this year we were delighted to hear that students at Highcrest Academy in High Wycombe had chosen the MPS Society as the charity they would be supporting for the year.

They kicked off their fundraising by holding a bag pack in their local Sainsbury's, where the students' hard work raised £158.26, as well as lots of awareness with shoppers!

Following on from this, Highcrest then invited the MPS Society to attend their Christmas Fayre to sell our merchandise, cards and raffle tickets.

A huge thanks to Ian Evans for all his help throughout the evening. We made a fantastic £91.30, and we would like to thank everyone who kindly donated and made purchases, and also to Highcrest for inviting us to the event. We look forward to more events with them in the near future.



### Card Making for a Good Cause

Dorothy Robinson (Nanny to Hannah Shannon - MPSIII) recently sent in a cheque for £100, which she raised by making cards - one of her lovely cards is shown opposite.

Dorothy has been supporting the MPS Society by making cards and holding card workshops for many years, so thank you so much to Dorothy and her friends for all their hard work.





## Skydive for Gene Therapy

Gary Harlock, dad to Jensen and Riley, who both suffer from Hunter disease, bravely took to the skies to raise money for gene therapy for MPSII.

Together with a team of friends Gary experienced the exhilaration of a skydive, jumping from 10,000 – 15,000 feet and falling at speeds of around 120mph. Not only did Gary find the courage to complete such a hair-raising fundraiser, but he also managed to raise a grand total of £6,695 (with more to come!) which will go toward gene therapy research and the work of the MPS Society. In addition to this, Jensen and Riley's mum, Jasmin is looking to add to Gary's total with a fundraiser of her own next year!

Thank you to Gary, James, Joe, Gideon and Tim for taking on the skydive challenge, and to Hefin and Bethan Richards who generously donated £620 of Gary's total as gifts on their wedding day.

If you would like to read more about gene therapy for MPSII take a look at the Research section in our Summer 2015 magazine (downloadable from our website) or turn to page 26 in this edition for a update on the research currently being conducted by Dr Brian Bigger and his team at the Manchester Centre for Genomic Medicine.

### *Skydiving for the MPS Society*

If the idea of an adrenaline-inducing skydive for the MPS Society gets your heart racing, why not get in touch and make it a reality? Our skydiving partners, UK Skydiving Adventures, make organising a skydive really easy. It is important to note that if you are doing a skydive for charity, you will be asked to raise a minimum sponsorship amount, which ensures that the costs of your jump are covered as well as reaching a target to make a difference to those suffering with MPS and related diseases.

It is safe to say that all of our supporters who have completed a skydive for us have had an exhilarating and memorable experience!

For more information visit <http://www.ukskydivingadventures.com> or call 01869 278706.





## Elliot's Lakes in a Day Ultra-Race

*In October, Elliot Moody took on his biggest challenge to date in the shape of a 50 mile non-stop race across the length of the Lake District, which raised a staggering £2,820 for the MPS Society. Read on for Elliot's account of this monumental fundraiser...*

After the huge success of last years 50 kilometre 'Thames Path Challenge' – it was always going to be a hard task going bigger and tougher this year. After weeks of deliberating, and I think in a moment of blind stupidity – I signed myself up for the 2015 Lakes In A Day Ultra-Race. An unsupported, unmarked 80 kilometre run from the top of the Lake District to the very bottom – the length of two marathons back to back, taking in some of the highest peaks including Blencathra and Helvellyn, to name a couple. What was I doing!? Each year since I've begun taking on these challenges, I'm always less expectant of raising a similar amount to the previous year, yet I'm always blown away and genuinely touched by the support, this year being no different! (I'm beginning to think people just enjoy watching me in pain!).

As the dreaded 10th of October race day approached, the nerves began to rise rapidly! The amount of well-wishes, good luck messages and donations from friends and family (and even people who had simply just heard about the challenge), really brought home the magnitude and realisation of what I was about to throw myself into. As this was an unsupported and unmarked race, where competitors would just be given an A3 route map and a head torch to make their way across the entire Lake District - I had planned to spend a few weekends in the build-up to the race, studying the route, the course, and things I would need to look out for and expect on the day. Unfortunately, eight weeks before the event, I was to suffer a torn knee cartilage which meant no training what so ever right up to race-day, with the injury not even fully

healed on the day. It was touch and go whether I should be racing – I probably shouldn't have been – but in my heart of hearts there was no way I was going to pull out.

The morning of the big day swiftly arrived and I found myself huddled up on the start line at 8am with around 300 other competitors. Many of them Europe's top fell-runners and ultra-running athletes, then, in the middle of them all, there was me! The phrase "all the gear, no idea" sprung to mind! Yet I was braced for what I knew would be a long, extremely tough day, and was prepared to have to draw on a fair amount of determination and dig deep! (little did I know how deep at this point!).

We set off out of tiny Caldbeck village, and instantly began climbing up towards the hills. The first section to Threlkeld feed station was 18km in length but what a baptism of fire! I was to first make the tough ascent up 'High Pike' trig point, before a slight drop back down where competitors were asked to cross a tricky stream – where in a comedy-like fashion, I slipped on a rock and ended up submerged from the waist down... quickly pulling myself up I thankfully realised there were no photographers in sight! Great start! From here it was another long, torturous, boggy climb to the top of Blencathra (at 2,800 feet up), before making the unbelievably tricky (and dangerous) descent down Halls Fell Ridge (where you're advised to basically do anything you can to avoid tumbling off the cliff face!) before arriving into Threlkeld village at midday. Here I would be met by my family, and friends

Emily & Sophie – and after explaining to them why I was white as a sheet, and how tough that first section was, the response from Sophie - "you'll be OK, you look like a ninja!" – thanks for that helpful input! The viciously steep climbs combined with the boggy swamp-like surface had been hell already, I had dehydrated massively and not been able to take on any energy for the whole section, due to the difficulty of the first few peaks. However, they turned me round, and after a quick re-fuel, I set off into the distance for the second section.

This section was to be even tougher. A long 28.5km stretch including the torrid climb up to the tip of Helvellyn at over 3,000 feet in the sky! During this stage, the field became stretched and I found myself running out of sight of any other competitors. I had managed to put a bit of distance between myself and the group of runners behind me, however the first of what was to be many navigational errors, coming away from Helvellyn, forced me to come back on myself to find the correct route, costing me around an hours time and a couple of miles extra distance! I found myself racing against the darkness to make the Ambleside feed station, and decided against opting for the head torch just yet. This proving slightly costly as during a down section I ran straight through a heavy bog, leaving one of my shoes completely behind – going back to get it, I found it was completely submerged in a pool of mud which I had to reach into, recover and squelch my foot back into! (This day just gets better!). The darkness did beat me and I reached Ambleside at 7.30pm after 11



and a half hours running. I was only half way and still had another marathon to complete! Again the welcoming faces and smiles of my friends and family greeted me outside the feed station and gave me a huge morale boost. At this point my legs, back and feet were throbbing with pain, my right-foot was beginning to blister with compensating for my opposite knee on the tricky down-sections, and frankly, heading out for another long 21.5km battle sounded like hell. Yet when you know someone living with the constant battle of MPS – you'll understand that it puts things into perspective, and gives you a determination to keep going no matter what. I took on a few bowls of pasta and other treats at the feed station, popped a few more painkillers, strapped on my head torch and headed back out into the darkness.

Somehow I managed to make good progress heading out of Ambleside and was now following the route down the side of Lake Windermere, after 28 miles of mountainous running, I was somehow managing to 'pick off' a few runners ahead of me – I felt in the zone, must have been the painkillers! Yet this wasn't to last for long...navigating through unfamiliar areas, through thick forest, woods and hills in complete darkness became nigh-on impossible. I made a number of navigational errors (as did many others) adding more frustrating miles and time. Much to my amazement, I later found out that for much of this section, I had been keeping pace with another competitor who had recently

completed the toughest footrace on the planet...250km across the Sahara Desert! My unfortunate lack of preparation was to soon to prove my downfall – and as I entered the early hours, being only agonisingly close to the final stop in Finsthwaite, and with my head torch now failing from the now 6 hours usage time in darkness, I made another huge navigational error and had to be put back on the correct path through communication from my uncle back at home in Yorkshire, who had sat up live tracking my progress into the early hours – as had many others!

Although it was 2.30am in the morning and at this point very cold – I was gratefully greeted into the final stop again by my parents, brother, sister, friends Caz & Gaz, Emily & Sophie, who had all been nothing short of unbelievable all day, I couldn't thank them enough. After an accumulated 52 miles, equivalent 44 race miles and almost 19 hours constant running – my feet were torn to bits, my head torch had given in on me and I was pulled from the race at the risk of serious injury. I was determined at this point to finish the mere final six miles but circumstance would prevent me from doing so. The errors in navigation had really taken their toll and cost me lots of time, mileage and damage to my already battered feet. I told myself I would never quit at any of these challenges no matter what the situation, so this decision hurt big time (and still does!)...yet I console myself that with the hindered training plan and carrying existing injury – I was happy with what I'd achieved.

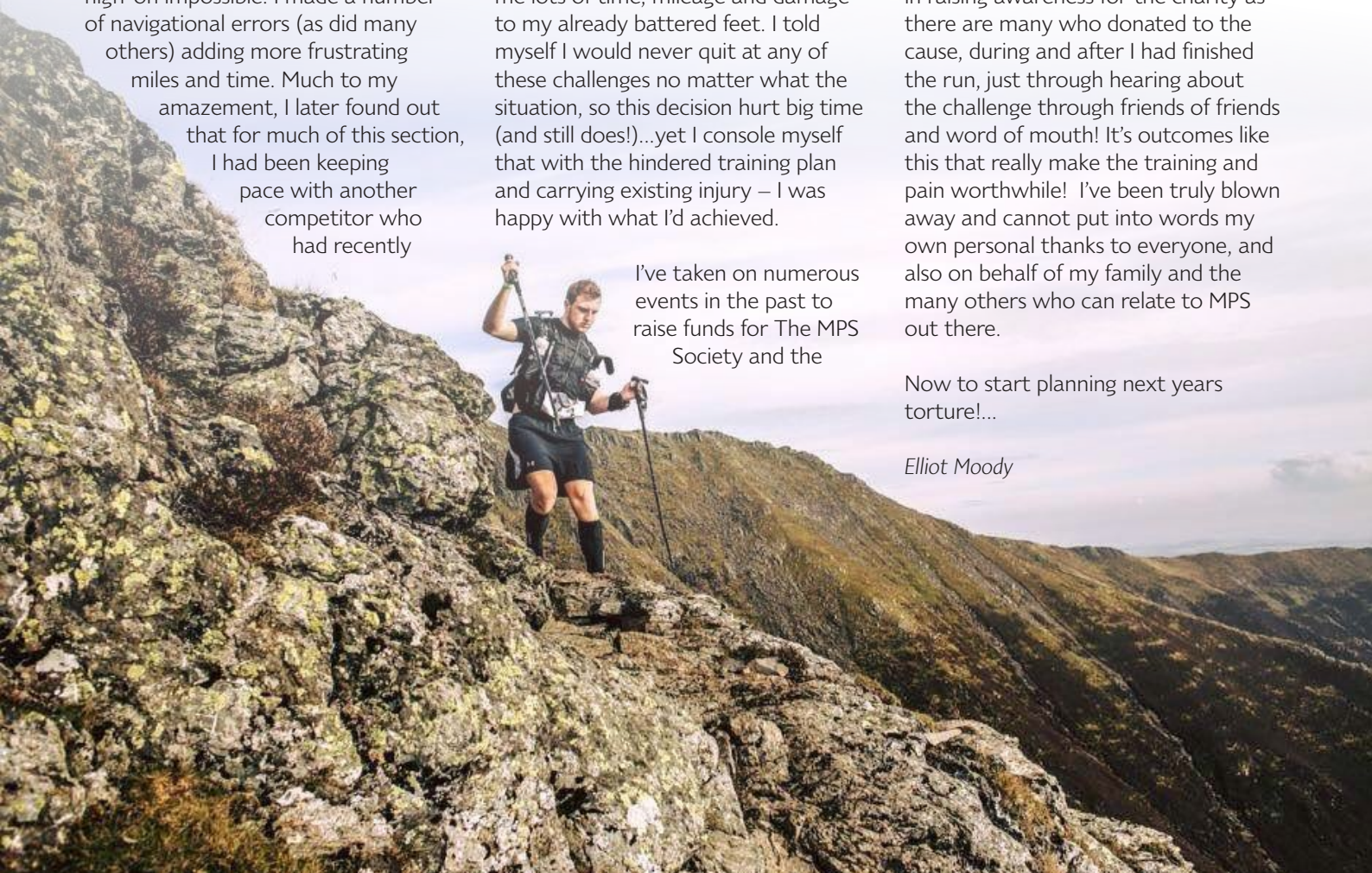
I've taken on numerous events in the past to raise funds for The MPS Society and the

support has always been incredible. Yet there was something extra special about the support for this one. In the weeks leading up to the day, and even on the Friday afternoon whilst driving up to the hotel – I was receiving messages and phone calls every few minutes, even from people I hadn't spoken to in months or years, wishing me well. On the day and throughout the event, I was arriving at the various checkpoints to hear of the many rally cries of encouragement, support (and concern!) through my family, who were having to post regular updates through social media! Then there were those (from down south, right up to Scotland!) who were tracking my progress from 8am in the morning to 2.30am the following morning live, through the dedicated website throughout the day and contacting me whilst I was running to put me back on the right route!

This was reflected on my JustGiving page – which at the time of writing this, has accumulated over £2,300 in sponsorship, through over eighty separate donations, and still rising! This is tribute to the many friends and family who continue to support this cause and donate their hard-earned money so generously every year. The event also seems to have done its job in raising awareness for the charity as there are many who donated to the cause, during and after I had finished the run, just through hearing about the challenge through friends of friends and word of mouth! It's outcomes like this that really make the training and pain worthwhile! I've been truly blown away and cannot put into words my own personal thanks to everyone, and also on behalf of my family and the many others who can relate to MPS out there.

Now to start planning next years torture!...

*Elliot Moody*







### *Wear it Wicked*

My name is Michelle Stark and in my small local community, Motherwell, I hosted a charity Halloween event called “Wear it Wicked” to raise money and awareness of MPS in memory and in honour of Luke MacDonald of Corby. A big thank you to his family for letting me raise money in his name! The night was very successful and everyone enjoyed it tremendously .....despite it being a family night and having loads of excited children there! We still managed to raise just over £800 - for such a small community I'm very appreciative.

*Michelle Stark*

*Thanks to Michelle and everyone who got involved to make the event such a success! The fundraiser even got into the local press, raising awareness in the North Lanarkshire and Glasgow area.*

## Fundraising Reminder

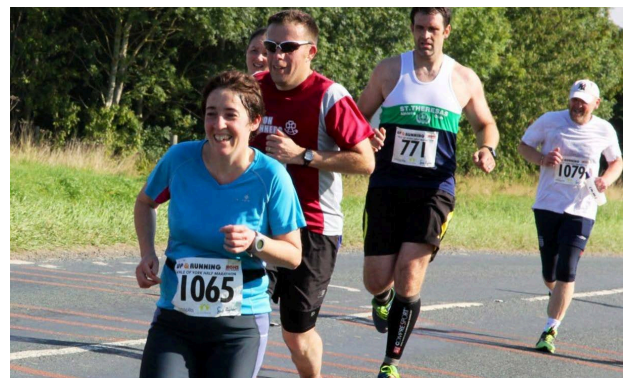
*When paying in your donation as a bank transfer, please remember to use your full name as a reference so we can link it to a fundraising event and pass on our gratitude.*



### *Fundraising Sisters*

Sisters Sally and Jenny Mitcham have raised over £4500 between them after Sally's son, Danny was diagnosed with MPS II earlier this year.

First up was Jenny who ran the Vale of York Half Marathon in September. Having run a marathon in 2014 she wasn't meaning to badger her friends for sponsorship again so soon, but as she says 'situations change and I felt I had to try and do something to help Danny!' She raised £1222 and in the process also managed to beat her personal best (set twenty years earlier), running the 13 miles in 1 hr 58 minutes.



Sally, however, hasn't done any running since school days, so had to come up with a different fundraising strategy. She chose to shave off her hair instead and also raised awareness of MPS when their story was featured in the local press. 'The response from friends and family has been tremendous,' she says, 'though perhaps doing it on the 1st November was a bit foolhardy. It's cold! Thankfully, people's generosity also stretched to the donation of a few hats for me to wear.' Her fundraising was added to by the staff and parents at Danny's nursery, a raffle held by the local childminders and an audience collection after the performance of a play Sally was in, giving her a grand total of £3,301.17!



### Great North Run

My beautiful sister Emma Jones ran the Great North Run for the MPS Society for my daughter Charlotte and husband Simon, both of whom have Fabry disease.

I cried when she sent me the photo of their names on the back of her running vest and I know she cried at various times during the run, thinking of them both too.

But the challenges they face, merely encouraged Em to run faster and she finished the half marathon in less than 2 hours! At the moment her fundraising is at £335 but I know that will continue to build, as she prepares for the Great Birmingham Run in October, which she is doing for the MPS Society also.

We are so proud of her and thankful too.

*Rachel Matthews*



### Ben Nevis Run

My name is Ross Brown and I was asked to write a short message about why I did my latest challenge. My wife Katie recently told me about her friend from work whose little girl Sophia was diagnosed with Sanfilippo syndrome.

When we looked on the MPS Society website I wanted to help raise awareness and obviously some money towards future research treatments and medicine. I decided to run Ben Nevis which I did in 3hours 10 minutes, up and down. I really enjoyed the run as its a good work out on the way up and a nice easy descent down. I managed to raise £1000.00 for the MPS Society which I was extremely happy about.

My next fundraiser will be the great Scottish run which is a half marathon, 13.2 miles. Next year I am going to run Ben Nevis again and run a marathon again for the MPS Society. I'd really like to help raise awareness as I feel that charity needs much more exposure and help.

*Ross Brown*

### Thank you for your Ink Cartridges & Mobile Phones!

Thanks to everyone who has sent in their old ink cartridges and mobile phones, we have so far raised a fantastic total of **£714.45**, just by recycling!

Please keep sending them in - the only cartridges that we cannot take are Epson, Kodak and those that have been previously refilled.







## Apollo Nine's Bake Sale & Fun Run

Hello! We are Apollo 9 and we completed our National Citizenship Service in the summer; a government run project for young people. As part of NCS we had to complete a social action project. One member of our team had personal connections with the MPS Society and suggested it to the group.

After long deliberations and plans we decided that the charity we would raise awareness and money for would be the MPS Society! The next challenge was to decide how we would accomplish our goal of raising £250 and awareness for this charity.

As a team of only eight people we were the smallest group and so we each had a lot to do in order for our events to work. First of all we arranged a cake sale at Northampton College which was a huge success. All of our team baked and bought a huge selection of goodies to sell. Coming away from that day gave us huge confidence as people were interested in what we were raising awareness for and they were very generous in their donations.

The main event that our team organised was a Fun Run! At Hartwell community centre on the field we set up a circuit for the runners and had some stalls to go alongside (for the non-runners). Getting sponsored to run and enter the race generated an amazing interest from family and friends. It was such a wonderful thing to get others to join in and take part.

After a very busy summer... Apollo 9 are exhausted! Nonetheless we all graduated from NCS and are now all starting at various colleges and sixth forms. The events we organised for the MPS Society were such a delight and we all got very passionate about this amazing charity. In total we have raised a fantastic amount of £412.50 for the MPS Society. We urge all of you to take part in fundraising for this cause, it is such a great charity.

*The Apollo Nine Team*



## Fundraising for Josh

This is Josh....

Josh is 6 years old and has Maroteaux Lamy Syndrome. Josh is the happiest little boy in the world and has never once complained about what he has to deal with on a daily basis.

He loves football, school and iPads, and he is the best brother to his 3 sisters. Josh is a massive Manchester United fan and any chance he gets he will be kicking a ball around until his little legs can't kick no more!

Right from the beginning of our MPS journey the MPS Society has been there. It was a very scary time for us not knowing what the future would hold for Josh and us as a family.

Just having someone on the end of the phone to talk to has helped in so many ways and I really don't think I could thank them all enough

The best way I feel I can give back is by raising as much money as I can for this amazing charity. So far we have raised just over £1,300 with help from our local community.

I would like to say thank you to everyone who works at the MPS Society. Each and every one of you are amazing.

*Sarah Cutler*





## Panto Fundraising for the MPS Society

Our family hobby is amateur dramatics and all of us are part of a group who stage a fun pantomime for charity every year, in February. This year we have the opportunity to combine our passion with support for two charities who have supported us over the years, and we will be donating half the profits from the 2016 show to The MPS Society. My children, Archie and Isaac, are a big part of the group and will be on stage again this time in “The Princess and The Peastalk”! Many of you will know Archie and Isaac from the MPS events and conferences and they would love it if any MPS families could make the trip to Gloucester to see the show.

Our group is called BADCo and is based at EDF Energy where we work – Dave and I have been involved for years, and Archie and Isaac started joining in as soon as they were old enough. They have played all sorts of characters in the productions, Archie having been in eight previous shows and Isaac in six.

In “The Princess & The Peastalk, Archie is playing the narrator and Isaac is the Deputy Court Jester, roles very well suited to their own character! The show tells the tale of Princess Lily who arrives at the Palace in the middle of a party after getting lost in the forest - naturally no one believes she is a real Princess and so the Queen decides to test her story with the traditional “pea under the mattress” test. No one was prepared for what happened to the pea – and Princess Lily - after that!

### Calling all knitters!

BADCo regulars will know that for several years, the “Knitting Grannies” have held a stall during our performances selling panto themed knitted goods to raise money for Age Concern (a BADCo charity a few years ago). Sadly they have now all retired from knitting and we will miss their presence.

However, BADCo would now like to continue in their spirit and run a similar stall for ourselves to raise extra funds for this year’s BADCo charities! So we need your help! CALLING ALL KNITTERS!!! In keeping with the Princess & the Peastalk theme, we are particularly looking for knitted

“The Princess & The Peastalk”, is presented by BADCo - The Barnwood Amateur Dramatic Company, from 25th to 27th February 2016 at EDF Energy, Barnwood, Gloucester, where we are part of the Sports & Social Club. As well as Archie and Isaac, the cast includes Dave and is written and directed by myself! The performances are Thu 25th Feb at 7.30pm, Fri 26th Feb at 7.30pm, Sat 27th Feb at 1.30pm and 6.30pm. If any of you can make the trip to support us and help boost the profits for The MPS Society, you will get a fun family friendly show with great song and dance routines, fantastic entertainment and a giant peastalk! Full information about how to book tickets and where to find us is on our website [www.badco.org.uk](http://www.badco.org.uk) and you can follow BADCo on Facebook too, to keep up with the latest news. You can also read about how you can knit for our charity stall too!

We have raised thousands for various charities over the years and we are really excited to be supporting The MPS Society this time. Archie and Isaac have Morquio (MPS IVA). As many of you are aware, this affects growth, resulting in short stature and so we are also supporting the Restricted Growth Association (RGA), which Archie and Isaac have been involved with recently. The show profits will be shared equally between the two charities. BADCo is a huge part of our lives and it will be great to share it with The MPS Society!

Anna Eaton  
(photos courtesy of David Sutton)

– or crocheted – pea pods, princesses, crowns, witches or jesters! Any other knitted gifts or toys very welcome too! If you can help then get in touch by emailing BADCo. [BarnwoodAmDramCo@gmail.com](mailto:BarnwoodAmDramCo@gmail.com) and we will try to share ideas, patterns and photos as we go along! Thank you in anticipation!

Anna Eaton







## Fundraising for Dylan

Dylan was born in September 2012 and he seemed a happy little baby, always smiling and he liked playing with his toys. We both became concerned for Dylan when he was 18 months old, he wasn't really doing what other children his ages were doing. He would struggle to walk without holding on to things, he wasn't talking just babbling a few sounds.

Clare spoke to the Health Visitor and explained her concerns about Dylan. We were referred to a paediatrician at our local hospital who checked him over and confirmed that Dylan had global development delay, he was referred for an MRI scan of his brain.

Dylan had his MRI scan under general anaesthetic and we had some blood and urine tests done at the same time.

A week before Christmas 2014 we were called into a meeting with our paediatrician. We walked into the room and there was a lot of medical staff in there. We were told that Dylan had MPSIII..... devastating news but we really didn't understand what MPSIII was.

Last Christmas was very difficult to get through as we were trying to keep positive and things as normal as possible for Clare's son, Callum who is now 7. It was such a difficult time for both of us. We told our family, which was probably the hardest thing either of us has had to do.

I had signed up to run the Brentwood Half Marathon in November 2014 and was going to run it to raise funds for another charity but as this had happened to us, I wanted to run for the MPS Society as they were the only UK charity able to assist my family with what we were dealing with.

I started training for the Brentwood half marathon on 2nd January 2015 and progressively built up my training regime over the next 11 weeks to enable me to complete the half

marathon. I work for LV one of the biggest insurers in the country as a Team Leader and a member of my team said they would run with me to help me raise vital funds for the MPS Society. I couldn't believe it, this person wanted to run 13.1 miles to help raise money for a charity that is so close to my heart.

Some of my team decided that a bake sale at work would be a great idea to kick-start the fundraising, so they arranged one for the end of January, which raised a staggering €307.52.

The morning of 20th March 2015 arrived and I was nervous; the hour in the car on the way to the start line I was petrified. I wondered whether I had done enough training, I wondered whether I would make it to the end..... I had to knowing that we had raised nearly €3,500 in sponsorship.

I finished my first half marathon in 2 hours and 11 minutes - such a massive achievement for me. Jack, my colleague was 4 minutes in front of me. I was pleased especially when some of my family were at the finish line cheering us on!

One of my colleagues and good friend, Sarah, decided that she was going to fundraise and walk 40km across the South Downs in May 2015, she showed amazing determination to do this and raised an amazing €500.

I then signed up for the Ipswich Half Marathon shortly afterwards which was in September, just thinking that it would be nice to do another one in 2015. To my amazement, several other people on my team and within LV said they would like to run for the MPS Society to support me. Clare and I were blown away by people wanting to help us and the charity.

*“Clare and I were blown away by people wanting to help us and the charity”*

As part of my work, I work closely with Enterprise Rent-A-Car, which is a global brand. Their relationship manager suggested I should apply for a donation from the Enterprise Holdings Foundation, the philanthropic arm of the company that, through its regional subsidiaries, operates the Enterprise Rent-A-Car, National Car Rental and Alamo Rent A Car brands. A cheque for £3000 was presented on 15th July and I was able to meet a lot of the staff at the MPS Society.

I completed the Ipswich Half Marathon in 2 hours and 25 minutes on the 20th September this year, and we had raised a further £1800 through sponsorship and a couple of cake sales.

LV contributed £1000 as part of their charity matching scheme as well, and the total far exceeded my original estimate. Overall, more than £10,000 has been raised and

donated this year for the MPS Society, with my friends and family looking for new fundraising ideas for next year.

Words cannot express our gratitude to the individuals named below and to our friends and family. They have all been amazing; both in the fundraising events that you have been involved in and the support you have given us. It is impossible for us to thank everyone but it is a heartfelt thank you...Jack Severy, Sarah Fournier, Clare Phillips, Mark Anderson, Kelly Wyatt, Zabeed Quddus, Tim Buttle, Laura Allenby, Sarah-Jane Hughes and The Enterprise Foundation.

Darryl Brook

(Photos from left to right: Sarah's 40KM Just Walk Challenge; Darryl & team at the Ipswich Half Marathon; Enterprise Holdings Foundation presenting MPS Society staff with a cheque for £3,000)

## MPS Charity Places for 2016

As many of you will be aware, each year the MPS Society purchases charity places for various country-wide events and we invite our supporters to fill them! Below is a list of the events that we have chosen for 2016, and each one promises a unique and memorable experience, plus a fantastic way to raise funds to help make a difference to families affected by MPS and related diseases.

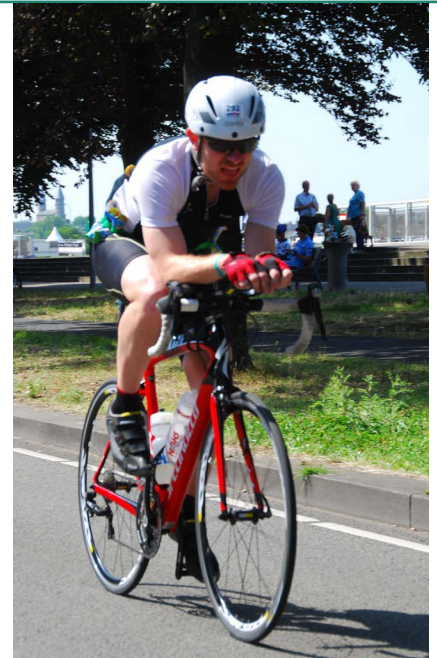
Our charity places for 2016:

- \* **Virgin Money London Marathon** - all our places for this event have now been filled, but you can register your interest for the 2017 marathon by emailing the address below
- \* **Vitality British London 10K** - Sunday 10th July 2016
- \* **Great North Run** - Sunday 11th September 2016
- \* **Great Manchester Run** - Sunday 22nd May 2016
- \* **Great South Run** - Sunday 23rd October 2016
- \* **Great Manchester Cycle** - date to be confirmed

We would love for you to challenge yourself and do something great for 2016, so if you would like to find out more please drop us an email at [fundraising@mpssociety.org.uk](mailto:fundraising@mpssociety.org.uk).

If there is an event that we have not purchased places for, it is still possible for you to enter in your own right, as many of our supporters did in 2015. If you do go down this route please let us know so that we can support you on your fundraising journey.

Please do note that if you wish to take one of our charity places that we will ask you to agree to raise a minimum amount. This is to ensure that the MPS Society does not lose money as we do have to pay the organisers for our places. Most of the time our supporters raise above and beyond the minimum, and we can provide you with all the materials and advice you need to do the same, but please do not accept the place if you are not confident that you can reach our minimum amount.





# MPS Society National Draw 2016

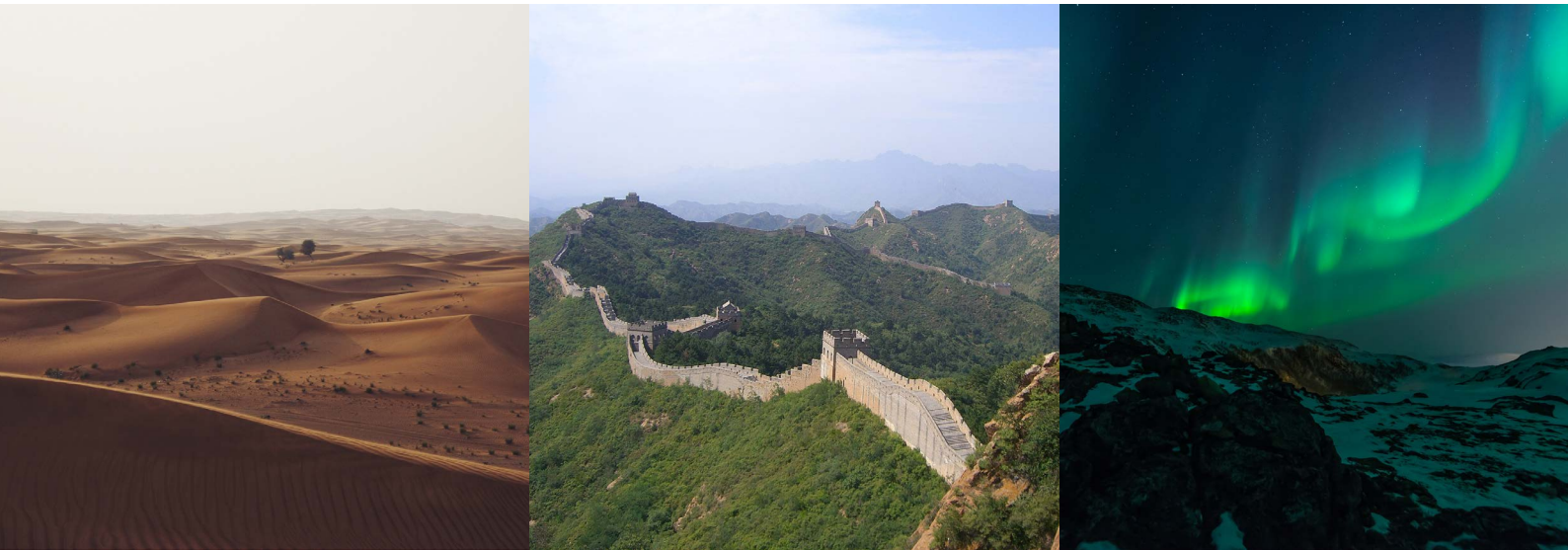
**Thank you to everyone who bought and sold our raffle tickets, and congratulations to our winners! This year our National Draw raised a brilliant £2,830.75!**

- 1ST: Three night family stay at the Adina Hamburg - Lee, 26387
- 2ND: £500 Millennium & Cophthorne Hotels and Resorts Voucher - Mr & Mrs B. Clarke. 09513
- 3RD: Three nights stay including Breakfast for two at Adina Hamburg - S. Jamil, 10774
- Pair of Wills to the value of £350 from Blandy & Blandy - Teresa Alvarez, 23112
- £300 M & S Vouchers - M. Copsey, 11160
- 'Life is Crawesome' photoshoot worth £400 - Robert Gibson, 11275
- £250 M & S Vouchers - Pat Gomm, 10004
- £125 Red Letter Day Voucher - Seedat, 31301
- Course of 10 sessions on Back in action Mobiliser System - S. Gremo, 00633
- £100 M & S Vouchers - E.M.A. Brock, 09953
- 2 tickets (& car park pass) to Cheltenham Racecourse New Year's Day - B. Marshall-Andrew, 15170
- £50 Voucher for experiencedays.co.uk - Culley, 00990
- £50 Ikea Voucher - Judy Dally, 28998
- Two Club Enclosure tickets for Royal Windsor 2016 Season - Jae Regan, 01292
- Anfield Stadium Tour for 4 people - Mrs Sue Murphy, 11521
- Family ticket to Circus-Starr Circus Winter Tour (any location in UK) - E.Jones, 02938
- Fitbit Flex Wristband - Mr K. Puddy, 21078
- Copy of signed photograph England Test Squad vs New Zealand 2015 - M.C. Dade, 30927
- £25 'one for all' voucher - P. Headland, 03989
- Kew Gardens entry for 2 people - Mrs P. Wray, 28829
- £20 Love to shop Voucher - M. Khaliq, 22897
- £20 Boots Voucher - R. O'Neill, 22707
- Personalised children's book from WondrousInk.com - S. Evans, 21236
- 2x Empire Cinema Guest Passes - S. Swayne, 31500
- £15 Amazon Voucher - Maureen Bird, 10981
- Cream Tea for Two at Van Hage Garden Centres Café - Paula Martin, 01672
- Kids Digital Movie Creator - Seedat, 31309
- GAME T-Shirts - Mrs S. Hill, 02153
- Personalised Children's book from PenWizard.com - Mrs Thomas, 08222
- Darcey Bussell Children's Book - Ian Mahoney, 03082
- Disney Store goody bag - Morven McLeod, 10811
- House of Fraser Scarf - K. Todd, 22083
- Assorted Cadbury Chocolate Box - Andy Orvis, 31837

**We were delighted to hear from one of our winners, Margaret Brock, who wrote in with the following:**

*I just wanted you to know how delighted I was when I opened your envelope to see that I had won the £100 M&S vouchers. I was so excited, I have never won anything of that value before, I couldn't wait to tell my family, especially my granddaughter, Hannah, and her Dad and Mum - Hannah has MPS.*

*They were so pleased for me. So thank you very much.*



## AIN'T NO MOUNTAIN HIGH ENOUGH

Fundraising is truly an individual activity - from bake sales and car boots, to head shaves and sponsored walks, the list of possible fundraising activities is only really limited by your imagination. For some, however, imagination involves challenge and adventure, and for these people fundraising has to be out of the ordinary.

If you are one of those crazy thrill-seekers wanting to raise your pulse rate while raising funds for the MPS Society, take a look at what Skyline Events has to offer (<http://www.skylineevents.co.uk/>). This company manage special challenge events, which includes the following overseas challenges:

- Sahara Desert Trek – 5th – 13th March minimum sponsorship £2,000
- Iceland Trek – 29th June – 3rd July minimum sponsorship £2,340
- Great Wall of China Trek – 8th – 17th September minimum sponsorship £2,700
- Mount Toubkal Trek – 21st – 25th September minimum sponsorship £1,620
- Petra Trek – 5th – 9th October minimum sponsorship £2,150
- Kilimanjaro Trek – 5th – 15th October minimum sponsorship £3,950
- Vietnam to Cambodia Cycle – 20th – 30th October minimum sponsorship £2,990
- Peru Trek – 2nd – 11th November minimum sponsorship £3,860

As you can see, these type of 'once in a lifetime' experiences do carry with them minimum sponsorship amounts, which cover the costs involved. However, with such an epic challenge you will probably find that it is not difficult to raise the funds needed, and we would of course be on hand to offer fundraising resources and advice.

If you are interested in finding out more about these special events, including both overseas and UK based challenges, please contact Skyline Events on 020 7424 5533 or email [enquiries@doitforcharity.com](mailto:enquiries@doitforcharity.com).





## *Thank you to all our donors including...*

**Mr & Mrs D Pottinger** donated £10 in lieu of a Ruby Wedding Anniversary gift for Jenny and Andy Hardy.

**Lizzy Farwell** donated £508.75 from running the Bristol Half Marathon in memory of her brother and sister, Katie and Tom, who suffered from Sanfilippo.

**Jenny and Andy Hardy** sent us a donation of £10 that they received from Mr & Mrs Pottinger that was given in lieu of a Ruby Wedding Anniversary present.

**Sarah & Jane Challand** donated £20 in support of Lizzie Farwell who ran the Bristol 10k.

**Iris Hitter** donated £230 raised from an open held in memory of Jacqueline.

**The Old Stein Lodge** raised £210 for The MPS Society from a charity raffle.

**Mrs Archer** donated £20 in memory of Mary Hood.

**Simon Farrow** ran The Great North Run for a second year in a row. This year he raised £425.

**Lara Tyler** raised £305 by completing the Great North run in support of Sam Brown.

**Robyn Cleghorn** raised £305 from the Nuts Challenge in support of Maddi who suffers from Sanfilippo.

**Lee-Anne and Eve** took part in the KM Colour run and raised £143.25.

**Sarah Fournier** raised £252.62 from the Just Walk event across the South Downs.

**Darryl Brook**, along with team members Kelly, Nicole, Mark, Zab, Clare, James, Bruce and Jack, all took part in the Ipswich Half Marathon and raised a team total of £1077.63.

**Carlos Da Costa** collected £140.45 in

donations from regulars in his local pub toward his fundraising for running the London Marathon.

**Tony Cuthbert** and friends provided an evening of entertainment in support of Will Brodie who suffers from MPS II Hunter Syndrome and raised a wonderful £900.

**Beau Brummel** in Seaham held a reunion for ex-employees and Pam Coils and Valerie Griffiths requested the monies raised go towards the MPS Society in support of Dominic Clark who has MPS I Hurler Scheie.

**Rick and Ann Coleman** donated £100 raised from an afternoon Garden Party held in Molesley, Birmingham.

**The residents of Bromley Street** E1 raised a wonderful £500 for the MPS Society in community fundraising events.

**Sam Barnes** raised £500 in support of his niece Sophia Scott who suffers from MPS III Sanfilippo by holding a BBQ and fun afternoon at the Boat of Garten Golf Club.

**Leanne Barwood** ran the Birmingham Half Marathon and raised £382.50 for the MPS Society.

**Andy Gas Southern Electric** challenged their contractors to a charity football match to raise money for the MPS Society. They held a raffle, cake sale and 'name the teddy' and raised a wonderful £594.45.

**Ian Evans, and Dennis and Janet Mauger** from Arriva The Shires raised a total of £502 from fundraising at The Halton Camp Fun Day and The Halton Village Festival.

**Berkhamsted Glee Club** held a concert named 'Calendar Days' at the Collett School in Hemel Hempstead and raised £1000 for the MPS Society.

They also created a 'Calendar Girls' style calendar and donated the proceeds of this to the Society. The total amount donated was an extremely generous £1666.66.

**Jen Taylor** organised a charity boxing event and raised £37.50 for Blake's Genes.

**Clare Moody** raised £275 from taking part in the Great South Run.

**Kelly Hadley** raised £435.17 by taking part in the Great South Run.

**Anne & Ken Hooper** donated a generous £3476 raised from a sponsored Fun Walk and Poughmans lunch in support of Jamie McFarlane.

**The Phoenix International Charity** donated £100 raised from Christmas card sales and various other fundraising activities. Rachel Capstick very kindly nominated us for the donation.

**Churchwood Primary Academy** held a Wear it Blue Day in September and raised £110.17 for The MPS Society.

**Manor High School** raised £668.25 on their MPS Awareness Day organised by Saffiya Seedat.

**Darren and Amanda Scott's** daughter Sophia suffers from Sanfilippo and they have raised £2500 from their "Hundreds of Promises" for the MPS Society.

**David Fowler** donated £816.47 by organising various fundraising events in Northern Ireland in support of Maddison Crockford-Reid who suffers from MPS III Sanfilippo. David also donated a further £60 from sales of MPS merchandise.

**The Purbeck Marathon Team** has donated £120 raised from The Purbeck Marathon and The Purbeck 16. They raised over £6000 in total and shared

## Donations

Mrs R E Willis; Mr & Mrs Shaikh; Sally Borrows; Ian Mahoney; Margaret Lyon; M Horsley; Zoe Warner; Mrs Belam; Janet Croft; Yunis Mohammad; Adele Jones; Deborah Irwin; E Mason; Pam Hope; Deborah Farrow; Mr & Mrs Shaikh; Mr & Mrs Vivier; Mr R Byrom; Norman Saville; Mr S Sefton; Mrs D Duckett; Carol Copsey; Mrs Puddy; Janet Gremo; Caroline Bell; Donatas Mikuta; Maureen Loveday; Pamela Coils; Mrs Baker; Sir Andrew Buchanan; Shirley Stewart; June Petty; John Challen; Antony Selwood; John Brown; B Simner; Peter Hawkins; Judith Swann; Wendy Turner; Susan Clarke; Kay Todd; F G Robinson; Louise Rudham; Christine Hume; Lauren Thompson; Jenny Quant; James Wise Charitable Trusts; Sue & Vic Lowry; D Jowett; Ann Todd; Avril Stokes; T McGinn; S M Ryan; Silvia Marsella; Mr J Croft; Peter Headland; Mrs E Hickman; Mrs A Belam; Robert Kenton; Mr D Mort; Andy Hardy; Barbara Watt; Mrs L Chambers; S Swayne; Janet Hillier & Family; Keith & Moira Darke; Mark Hughes; Kevan Gelling; Mrs V Zaldua; Richard Errol Jones; M H Crespin; Bryan Winchester; Jasmine McLaren;

Marlene Murty; Jackie Smith; Chris & Julie Kembrey; Sharon Yates; Sally Mitcham; Mrs M Thomas; Saima Javed; Sam Barnes; J S Downes; C & B Newson; Michael Edwards & Louise Bee; Michelle Wood; Kathryn Wallis; Howard & Linda Matthews; friends & family of William Reeves; Elaine Bertram; Rosemary Pointon; Clare Walker; Nigel Lever; Sue & Vic Lowry; Sally Mitcham

## In Memory

Jack Stuart; Mrs Florence Grove; James Carson Jamison; Peter Lavender; Joan Colvin; Anabelle Shepherd; Gracie Bella Sims; Marjorie Hill; Marjorie Banks; Deirdre Ann Coles; Daniel Allen; Denis Rowan

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

Kathryn Wallis; Ellen Graham, Your Derwent & Solway Housing Association

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

J & F Winzar; J Casey; E M Lee; K & JB Hudson; Daniel Winzar; J & V Hastings; Marcia Tosland; S Littledyke; Norman Saville; S Bhachu; C Cullen; S & F Brown; I & A Hedgecock; V M Lucas; S Winzar; D Forbes; P Shrimpton; William Cavanagh; Barbara Harriss; L Brodie; M Malcolm; J Dalligan; E Mee; K Brown; E Moody; M Hahner; A Sabin; E Brock; M Fullalove; E Parkinson; G Ferrier; M Leask; R Taylor; R Gregory; A Ephraim; L Stillwell; K & S Bown; S & J Home; V Little; M Reeves; Z Gul; S & D Greening; D Palmer; M Parmoor; R Dunn; M Newell; C & M Gibbs; Mr & Mrs Cock; A Dickerson; M Kalsi; P Summerton; A Weston; E White; C L Hume; A Sullivan; N Cadman; J Wilson; J & M Wood; A Tresidder; E Cox; A Byrne; M Rigby; K Robinson; Mr Thompson; K Osborne; D Robinson; M Peach; R Arnold; J Ellis; I & V Pearson; R & J Richards; G Simpson; R & K Henshell; A Lockyer; Neil Upton; J Heritage; Mrs K Wallis; Nick Miles; Paul Berg; Elizabeth Merryweather; J. Garthwaite; Abby Thomas; Michael Morris; Tim Peach; Peter Rennoldson; Matt Mould; Tmara Senior; Amanda Laycock

this between charities nominated by their volunteers. The MPS Society had 4 nominations, which we are very grateful for.

**Cheryl Gillan MP** took part in a research survey for YouGov and requested the compensation of £80 was sent to The MPS Society.

**Marina & Friends** donated a wonderful £4654.74 for MPSIII research from the sale of second hand items in their shop.

**Anne Cotton and Heather Gordon** recently held a fundraising golf day at St George's Hill Golf Club, where they raised an unbelievable £16,379! Many thanks also to the Solo Charitable Trust for contributing £180 to that total, as well as to everyone who gave so generously at the event.

**Dorothy Taylor** held a fundraising event at her salon in Southport in support of Josh Westhead, who suffers from Mannosidosis, and raised £180.

**Iris Hitter** held a quiz night and raffle in memory of her daughter, Jacqueline, and managed to raise a fantastic

£152.22. Iris wrote in to tell us that during the course of the night they also received an additional surprise donation of £150 from a gentleman from the Taylor High Memorial Fund, who happened to be in the pub and won a couple of the raffle prizes!

**Saima Javed** did a sponsored skydive and managed to raise an amazing £549.44. Well done, Saima!

**G Stuart** donated £20 in memory of Jack Stuart on Jack's 21st.

**Carol Westland and Joan Phippard** sold handmade crafts and gifts throughout 2015 and donated their proceeds, which amounted to a fantastic £300.

**Jane Roberts**, who has worked at the Willink for 15 years, recently celebrated a 'big' birthday and very kindly asked for donations to the MPS Society in lieu of presents, and raising an amazing £275. Thank you, Jane, and Happy Birthday from all at MPS House!

**The Terry School of Dance** in Renfrewshire donated the proceeds

from their ballet school show, which also included a raffle. The school raised £500. MPS Society Trustee, Judith Evans helped with the sale of the tickets and even performed the can-can with the adult jazz dance group!

**Elizabeth Hull** completed a 10K run at Weston Park and raised £477.86 on her Justgiving page.

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



