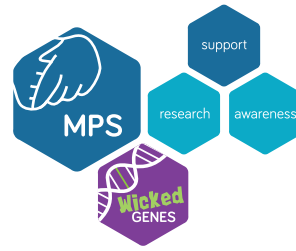


THE MPS MAGAZINE



**Society for
Mucopolysaccharide
Diseases**

**Support
Research
Awareness**

Autumn 2015

www.mpsociety.org.uk

Legoland Family Day

Back in August we held a family day at Legoland. Some of our families wrote in to let us know how the day went for them, and you can read their comments on pages 16-17.

4th ISMRD Glycoproteinoses International Conference.

Shirley and Sam travelled to St Louis, Missouri, USA to attend the conference where they met other families and professionals. Shirley's report can be found on page 18

Glasgow Women's 10K

No less than 53 ladies took to the streets of Glasgow to raise funds and awareness for the MPS Society. Altogether Team Glasgow raised over £11,470.38!

Read more on pages 32 - 33



Chloe (MPSI) and her family at the Legoland family day. Read more about the day on pages 16-17

Front cover photo: Chloe Blanc (MPSI) & family at Legoland

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magazine@mpssociety.org.uk

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

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Contents

04 × Chief Executive's Report

05 × Vimizim Campaign

06 × Governance and What's On

07 × Announcements

08 × Advocacy

13 × Remembrance

16 × Events

18 × International

19 × Research and Treatments

24 × Information & Resources

25 × Fundraising

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.

Once you have read this magazine, please pass it on to your family, friends and colleagues.

Help us spread the word about MPS and related diseases.

www.mpssociety.org.uk



facebook.com/mpssociety



twitter.com/MPSSocietyUK

Regulating Fundraising for the Future

A report by Sir Stuart Etherington Chair of the Review of Fundraising Self-Regulation

It has been a difficult summer for charities. Negative media coverage has been to the detriment of us all. The stories picked up on failings in a relatively small number of charities, but tapped into a much wider, much stronger, public concern about how charities ask for money. Criticism has rained in from all sides and I think many would agree that this cannot and should not continue.

This summer, I chaired a cross party review of fundraising regulation for the Minister for Civil Society. During the course of our review we have heard many voices and read many concerns, whether for the donors that give or for the future of the charities whom we hold so dear. I believe that we have been given clear signals as to the future direction of fundraising and the checks and balances that should remain in place around it.

First, and most importantly, we have heard that trustees must know what fundraising activities are taking place in their name. The crisis of trust we face is not simply a fundraising issue, it is a governance issue. Our review has therefore concluded that trustees need a clearer view and a stronger hand on the fundraising tiller

Secondly, charities and other

fundraising organisations need a new, more visible and effective fundraising regulator. The public have to know where to report their concerns. We are appreciative of the work that the Fundraising Standards Board has done, but we believe that the confidence of the public requires a new approach. We have concluded that a new regulator with stronger powers to sanction, universal coverage and better resources is a necessity. This includes the establishment of a new Fundraising Preference Service so that the public can easily stop unwanted fundraising communication from charities.

We have also concluded that the Institute of Fundraising should hand over responsibility for setting the rules which govern fundraising – the Code of Practice – to the new regulator. One of the most important issues to emerge from our review is that the public interest overrides any professional interest. Nothing less than handing over responsibility to the new regulator for setting the rules will do.

Fundraising regulation at present is unnecessarily complex – too many bodies with competing interests. We have recommended that the Public Fundraising Regulatory Association should merge with the Institute of Fundraising, but that its regulatory

responsibilities should move to the new fundraising regulator. Better regulation requires a clear separation of roles and interests.

We heard a clear message from charities and the government that there is little appetite for state regulation and all that this entails. If we are to maintain the trust of the public we must be able to show that we can regulate ourselves. Nevertheless, some breaches of the rules on fundraising may raise concerns that there has been a breach of trustee duties. In such cases, the Charity Commission can and should investigate. And we believe that reporting to parliament – not the government – provides for both accountability and independence.

I am cautious of statements that this review, and other activities in the fundraising world, represent a turning point or the crossing of a Rubicon. They do, I hope, represent the start of a long hard journey to rebuild public confidence in fundraising regulation and trust in charities.

With every best wish,

*Sir Stuart Etherington
Chair of the Review of Fundraising Self-Regulation
NCVO Chief Executive*

MPS Society Fundraising - Our Pledge

In the light of Sir Stuart Etherington's recommendations to Government and the need for more rigorous regulations for the benefit of the public and charities, the MPS Society has no trust in self-regulation and the role of the Fundraising Standards Board (FRSB) and will be discussing discontinuing the Society's membership of FRSB, including removal of the logo from publications and the website going forward.

The MPS Society never has and never will be involved in cold-calling, 'chugging' or passing our supporters' details on to data firms.

Our pledge is to always respect our members and supporters, and to uphold our good reputation.

If you would no longer like to receive communication from us, please let us know by calling 0345 389 9901 or emailing mps@mpssociety.org.uk and we will remove you from our database.

On the other hand, if you wish to hear more from us, remember to follow us on Facebook and Twitter.

*Christine Lavery
Group Chief Executive*

Welcome

This edition of the MPS Magazine contains all the usual up to date information and news concerning those affected by MPS, Fabry or a related disease, from research and treatment to advocacy and clinics. As always we also have a packed fundraising section, full of your fantastic fundraising stories, plus some great ideas on how to support the MPS Society.

Back in August we were delighted to be able to take a group of families to Legoland, which proved a real hit for children and adults alike. You can read their stories on pages 16 - 17.

Thank you to everyone who contributed to this magazine. If you have a story to tell, please get in contact by emailing magazine@mpssociety.org.uk.

Best wishes

The MPS Team



Chief Executive's Report

Christine Lavery

After a rollercoaster 16 months described as playing snakes and ladders and championship chess at the same time children and adults with Morquio disease and their families finally had something to celebrate when on 2 September 2015 NICE issued new guidance giving a YES to the reimbursement of Elosulfase Alfa (Vimizim) in England. The 'YES' is not straight forward and is subject to the MPS Society, BioMarin, NHS England and MPSIVA clinical leads in England agreeing a detailed and robust managed Access programme. The NICE approved managed access programme will also require compliance by those receiving reimbursed Vimizim as NICE require that ongoing data is collected over the next 5 years for evaluation and review. The disappointing side of this news is that NICE's results of its final consultation are likely to delay reimbursement by NHS England by some months. Whilst those who were on the clinical trial are now back on free Vimizim the children diagnosed since the clinical trial closed continue to deteriorate whilst waiting for treatment with Vimizim but hope is on the horizon.

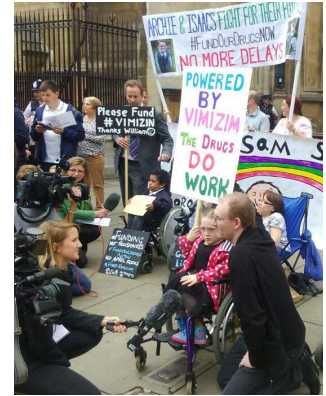
North of the border in Scotland 4 children and 3 adults with Morquio disease and their families are literally reeling from the news on the 6 September that the Scottish Medicines Consortium (SMC) have decided NOT to recommend

Vimizim for reimbursement in Scotland. Unlike England where the MPS Society supported its members to sustain a Massive 16 month campaign the Scottish members had falsely been led to believe that the SMC were likely to approve reimbursement of Vimizim and therefore families decided to trust the process which on reflection is deeply flawed. It has been touching to see Morquio children in England supporting and consoling their friends with Morquio disease in Scotland where the battle is just beginning.

We have chosen to showcase the Morquio campaign over the past months because the battle for Vimizim is just the tip of the iceberg. We are undoubtedly in a new world with NHS England with all ultra-orphan drugs for LSDs receiving marketing approval by the European Medicines Agency (EMA) having to be appraised by the NICE's Highly Specialised Technologies appraisal process. The next drug is for Lysomal Acid Lipase (LAL D). Going forward new therapies for MPSI, MPSII, MPSIII, MPSVI, Mannosidosis and Fabry will mean many more children and adults being at the mercy of the NICE process.

Christine Lavery
Group Chief Executive

Vimizim Campaign



We have had a very turbulent few months with significant announcements in England and Scotland.

Following NHS England's decision not to make a decision on 2nd July, treatment stopped for the ex-clinical trial patients. Devastating is an understatement.

We hosted a very successful event in Parliament attended by a number of MPs, Peers, clinicians and patient organisations to discuss the future of access to treatment for individuals with ultra-rare conditions.

Following our very civilised event Greg Mulholland MP attempted to raise the catalogue of NHS England's errors in Parliament, which due to not being succinct, led to abhorrent behaviour by the speaker, John Bercow. With the support of The Sunday Express who have followed our campaign Sam Brown successfully made it on to the front page!

With drug stopped the clinicians swiftly arranged that ex-clinical trial patients would come to the hospital every four weeks for a number of clinical tests so that any changes to coming off treatment could be measured as evidence for NICE. It was suggested that parents and individuals also monitor any changes to evidence the necessity of treatment.

We held another huge protest outside Parliament, attended by around 50 members and their friends and families. The BBC and ITV/Granada interviewed for a number of regions and several MPs came out to meet with their constituents and be interviewed for

their regional television. The BBC were keen to help us and re-iterated the importance of filming regularly to demonstrate any changes which they would be happy to air.

Following some significant behind-the-scenes work, free drug was re-started to ex-clinical trial patients.

The MPS Society met with NHS England along with Greg Mulholland MP and the other patient organisations who we have been working closely with to explain the frustrations and failures in more detail. Following that meeting we have been communicating in pushing for homecare. This week written confirmation has now been provided by NHS England and homecare should now be available to patients. This is a small but significant win and life-changing to those who were still travelling hundreds of miles for treatment.

Back in July we also had what we thought would be our final NICE meeting which would lead to a final decision on 28th October. Our patient experts did phenomenally, well speaking on behalf of the whole Morquio community and it was to squeals of delight that NICE announced that they are leaning towards a 'yes' decision which is fantastic.

However, the 'yes' came with the condition that there is a Managed Access Agreement. This is the first time NICE have looked to a more flexible approach to agree a decision and there will be a number of people who will be vocal against this. We are only a slim margin away from a 'no' and so

welcome this opportunity. We have been working incredibly closely with the expert clinicians, NHS England and BioMarin to create this managed access agreement. If NICE deem this agreement to be robust enough they will make their final decision a positive, in January!

All the stakeholders involved in creating this document want the decision to be positive and are committed but we are once again disappointed that the timescales have been extended further to January.

Whilst England has been following the NICE process, Scotland made a final decision not to fund elosulfase alfa, licensed as Vimzim. This decision came a week after NICE's leaning to a yes so was a complete blow to all our Scottish families. Within 24 hours of the announcement, and after a number of letters had been sent, First Minister Nicola Sturgeon wrote saying that there are systems in place for prescribing on an individual basis. We are continuing to put as much pressure on as possible to make sure that a positive decision is made to ensure funding for individuals in the future.

Both Wales and Northern Ireland look to NICE's decision and will hopefully follow with a yes.

Please do follow us on Facebook and Twitter for updates over the next coming months.

*Charlotte Roberts
Communications Officer*

News From the Board of Trustees

The Society's Trustees meet regularly. Here is a summary of the main matters discussed and agreed at the Trustee Board Meeting on the 10 - 11 July 2015 at MPS House, Amersham.

Governance

The Board had a discussion regarding appointing two new Trustees. In considering the skill base of the current Board it was agreed an article should go in the October 2015 MPS Magazine inviting letters of interest from Members with the following skill bases: human resources and accounting.

Susan Peach stated that she had received all but one completed self-appraisal form

Personnel

The Board welcomed the Internship initiative this summer and expressed a wish to see such opportunities offered in the future. The Board unanimously agreed the legal advice received that the post of Chief Executive Officer become the Group Chief Executive Officer for the MPS Society and MPS Commercial. The Board also agreed the MPS Group Staff Organisational Structure.

Advocacy Support

Sophie Thomas, Senior Advocacy Officer, gave the Board of Trustees a 40 minute presentation on the work of the Advocacy team. The presentation was very well received and gave Trustees a clear insight of the breadth and depth of the advocacy support provided to member families.

Access to Medicines

The Board were fully appraised of the progress made in the Vimizim and acknowledged the role Charlotte Roberts has played in the Campaign to date. The Board of Trustees approved the crowd funding initiative in support of the MPSIVA Campaign. It was agreed to invite Charlotte to present on the Vimizim Campaign at the September meeting.

Policies

Tim Summerton confirmed he is still waiting for three Trustees to respond to the Charity Commission Financial Controls Policy questionnaire. It was agreed Tim will present his findings to the Board of Trustees at a future meeting. It was agreed the Finance Officer and the Group CEO answer the questionnaire with the correct answers.

Annual General Meeting

The AGM was held at 11.30am on Saturday 11 July 2015. Eight Trustees were present and three offered their apologies. The Group CEO was in attendance. The Minutes of the AGM held 5 July 2014 were agreed unanimously and duly signed.

It was agreed that the AGM for 2016 should take place in February at the same time as the accounts are reviewed and agreed.

The Board agreed to appoint McLintocks as auditors for the MPS Society's accounts year ending 31 December 2015.

Judy Holroyd and Susan Peach Directors retiring by co-option in accordance with the Company's constitution were elected as Directors of the Company. Paul Moody, a Director retiring by rotation in accordance with the Company's constitution was re-elected as a Director of the Company.

What's On

MPS Regional Clinics 2015

MPSI - GOSH:
22nd December

MPSIII - GOSH:
8th December

MPSIV - GOSH:
13th October

Fabry clinic - BCH:
23rd October

MPS clinic - BCH:
27th November

MPSI Post HSCT (over 6 years) - RMCH:
16th October

MPSI Post HSCT (under 6 years) - RMCH
23rd October

NI children's MPS clinic - RBHSC
20th November

NI adult MPS clinic - Altnagelvin
14th October

NI Fabry clinic - RVH
15th October

Conferences and Regional Events

Childhood Wood planting
25th October 2015

**Manchester Family Day
Jack & the Beanstalk pantomime
Cophthorne Hotel**
28th November 2015, 12 - 3pm

**Liverpool Family Day
Beauty & the Beast pantomime
Atlantic Tower Hotel**
29th November 2015, 12 - 3pm

**Northern Ireland Christmas Party -
Dunsilly Hotel, Antrim**
17th December 2015

**Scottish Information Day -
Beardmore Hotel, Glasgow**
20th January 2016

**Welsh Information Day -
Holiday Inn, Cardiff (North M4)**
28th January 2016

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

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

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

Announcements



Rosemary & Harry



Lisa

Happy Birthday Lisa & Harry!

Lisa Nurse (MPSIII) recently celebrated her 44th birthday in a joint event with her dad, Harry, who was marking his 80th. Lisa and Harry enjoyed a garden party with family and friends, and a good time was had by all!

A big Happy Birthday to Lisa and Harry from all of the MPS Society team, and thank you to Rosemary Nurse for letting us know and sending us some great photos from the day. As Rosemary said in her letter to us, reaching her 44th birthday is "a great achievement in our daughter's life".

Result!

Congratulations to Naomi Carter, who recently received two As and two A*s for her GCSEs in English, Science, Latin and Classical Civilisations respectively.

Naomi, who suffers from Fabry disease, lost her vision a week before her exams, but still managed to gain this tremendous result - well done!



New Members:

Hamda has recently been in contact with the Society. She has a diagnosis of Scheie disease and is 15 years old. The family live in the South East area.

Laura has recently been in contact with the Society. She has a diagnosis of Fabry disease. The family live in the North West area.

Sally and Craig have recently been in contact with the Society. Their son Danny has a diagnosis of MPSII Hunter disease. Danny is 3 years old. The family live in Yorkshire.

Paul Cully has recently been in contact with the Society. His son has a diagnosis of Hunter disease. Ethan is 6 years old. The family live in the South.

Michelle and Spencer have recently been in contact with the Society. Their daughter has a diagnosis of MPSIIIC Sanfilippo disease. Maddison is 7 years old. The family live in the South.

Mr and Mrs Amer have recently been in contact with the Society. Amalia has a diagnosis of MPSIV Morquio disease and is 3 years old. The family live in the North West area.

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
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

- 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



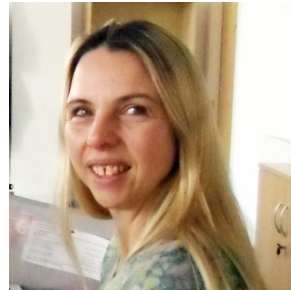
STEVE

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
MLII

Wizzybug Wheelchairs

As many families are aware, mobility can be an issue for children affected by MPS or a related disease, and in many cases mobility aids, such as wheelchairs do have to be considered.

If you have a child aged under five years old, you may want to look into Wizzybugs, which are wheelchairs specifically designed for young children. As there is no NHS funding for powered mobility for children under 5, the company fundraises so that they can provide Wizzybugs on loan to families for free.

If you would like to learn more about Wizzybugs, take a look at their website: <http://www.designability.org.uk/product/wizzybug/>



Bereavements:

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

Joseph Tilling who suffered from MLII I-Cell disease and passed away in February 2015 aged 6 years.

Lewis Broadley who suffered from MPSIII Sanfilippo disease and passed away on 12th February 2015 aged 17 years.

Nicolina Stangoni who suffered from MPSIV Morquio disease and passed away on 29th June 2015 aged 55 years.

Martin Beecroft who suffered from Fabry disease and passed away on 3rd August 2015 aged 55 years.

Sania Hanif who suffered from MPSIII Sanfilippo disease and passed away 8th August 2015 aged 13 years.

Joe Tucker who suffered from MPSII Hunter disease and passed away 20th August 2015 aged 19 years.

Clinics

GOSH MPSI clinic 14th July 2015



Abigail

It has been a few years since I have been to the GOSH clinic as it is usually Debbie or Steve that attend. So much has changed from the nursing staff to the doctors but it was lovely to meet everyone. The families started to arrive and joined in the noise and play of the waiting room. Abigail was the first to arrive and she had a great sparkly 'Animal' t-shirt.



Sienna

Hannah was very occupied with her i-Pad and just wanted to get out of there! Sienna had grown so much since I saw her a couple of years ago and was very shy. Last but by no mean least was Sophie who could talk for England.



Hannah

All the families have some fab holidays lined up in France, Spain and even San Francisco...

See you all again at the end of the summer.

*Rebecca Brandon
Advocacy Support Officer*



Sophie

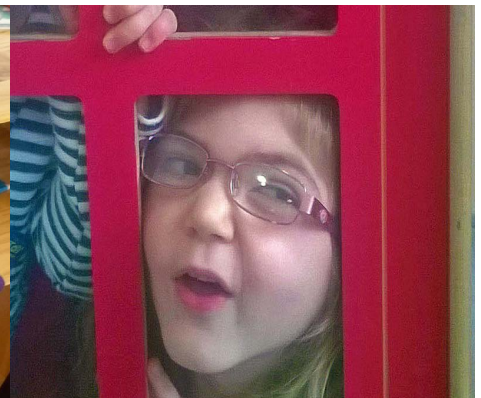
Royal Manchester Children's Hospital MPSI clinic July 2015



Jake



Leighton



Miya

GOSH MPSIII clinic 8th September 2015



Patryk



Pranav



Daniel

Our Experience at the Family Clinic

My family was diagnosed with Fabry disease 7 years ago and we have always had our check-up appointments separately - meaning different dates, different hospitals, more time off work/school and more expense! In August both my children and myself all travelled to London to visit the Royal Free family clinic. My adult consultant, Dr Hughes, and the paediatric consultant Dr Ramaswami were present.

I thought that a family clinic was a great idea and worked for us as a family. What I found good about this was that we were all seen at the same time, at the same hospital which was less stressful for us all as these checks are not much fun! We had our checks carried out that day which was a full



and busy day but we still made time for a bit of sight-seeing. Having a family clinic really made a difference to our checks - and now we know we are all okay for another year! I know we are in the best possible hands but even more so with two fantastic consultants.

Kristy Lyons

Congratulations to Chris Hendriksz on his Professorship

Chris Hendriksz is a Consultant in Transitional Metabolic Medicine and Clinical Lead of the Adult Inherited Metabolic Disorders department at Salford Royal NHS Foundation Trust, Manchester, UK. Before that he spent 8 years at Birmingham's Children's Hospital, so many of you would have seen him, or even heard him speak at one of our events.

We were delighted to hear that Chris was awarded a well-deserved Professorship back in 2014 and he kindly wrote in to tell us more.

I have been working and helping the University of Pretoria since 2011. In August 2013 I agreed to help set the program and bring international speakers to Pretoria for the first rare disease symposium for doctors and allied health care professionals. This was hosted in November 2013 and the whole program was recorded and then made available for clinicians throughout Africa; we have 450 subscribers to date. I also agreed to help manage difficult patients and we had our fist clinic after this event. Since then I have had a few more clinics there and I am available for the clinicians and allied health care providers all the time to help with difficult cases or help them get treatment. I have helped them to develop some national guidelines and also to host follow-up events. I also became involved in marking student projects and giving some advice on projects both at University of Pretoria and some of the other universities in Southern Africa. I also agreed to help with the next symposium to be held in November 2014 and again bring a team of experts out.

In May 2014 I was nominated to become an extraordinary professor of the University of Pretoria in recognition of my contribution to the university. I studied at the university and was regarded as an international contributor to academic knowledge, with more than 90 peer reviewed papers. I have returned to help further services for patients with rare disorders in Southern Africa and have supported the advance of knowledge in collaboration with the university. In August



2014 I was offered the position and at the November 2014 meeting it was officially announced to all the delegates and I accepted to make my role formal. I felt very honoured but didn't use the title and it was only when I had my heart attack that it changed. I went for counselling and as part of my rehabilitation program I was encouraged to learn to celebrate my achievements as that would help with stress management and my overall health. In short that was the reason why I informed every one firstly on LinkedIn in February and started using my title once I returned to work. This work will continue and I am already planning the next symposium in 2016.

I am trying to look after myself as best as I can and also realise that I can't do what I used to but that doesn't take my drive away to do what is right for patients. It is a pleasure to help with NICE and ultimately I have to support this until it is all done and patients have access to treatment; this would be impossible without the help of the MPS Society. Once that is done I will slow down even more as I hope to be around quite a few more years but at slower pace with more focus on the things close to my heart. Patients will always be part of that.

Chris Hendriksz

All Ireland Advocacy Support Update

We are coming to the end of what has been a fairly wet and chilly summer over in Ireland. I hope that you've enjoyed your time off school and work and are all set for the year ahead.

Since I last updated you on the All Ireland Service we've had a busy time – spending lots of time travelling around Ireland visiting new families, meeting with housing departments, preparing care-plans and working closely schools as everyone make preparations for the incoming academic year.

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

In my last article I let you know all about our 20th anniversary meeting in Northern Ireland and already we're panning our next big events. We hope to be able to welcome as many of you as possible to these events which promise to be both fun and informative!

Alison Wilson
Advocacy Support Officer

Northern Ireland Christmas Party Thursday 17th December Dunsilly Hotel Antrim



I know that people hate to wish away their year, but here at the MPS Society we're already planning for the festive season! We would be delighted to welcome all of our Northern Irish families along to an evening of festive fun and entertainment.

This year our party theme will be **'The Sights and Sounds of Christmas'** and we promise a night of sensory experience for the whole family!

Upcoming Events

The Sights and Sounds of Christmas

This year our Christmas party will have a new twist. The party will be focussed on the sights and sounds of Christmas. We plan to bring you a magical experience for all ages and hope as many of you as possible can attend. If you have any questions please do not hesitate to get in touch with Alison.

Save the date - All Ireland Conference

We are already making plans for our biennial All Ireland Conference at the Hilton Hotel in Templepatrick in 2016. It was lovely to see so many of you there last year and we hope to make next year even bigger and better. If there are any topics that you would like to see included on our programme please get in touch with us and let us know. This is YOUR conference and we want to make it as valuable as possible for you and your family.



Remembrance



Nicolina

A very bright light has gone out in the Stangoni family. Nicolina was a little person with a fantastic sense of humour and warmth. You just couldn't fail to love her.

Nicolina was born on 11th April 1960. She walked, talked, and even joined tap dancing classes and the Brownies as any other little girl would do.

Nicolina attended the village primary school from the age of 5 to 11 years, and it was when she was about six years old that she complained of pains in her knees. Dad would have to rub her knees before she could walk across the playground. After seeing a GP, he said it was probably just growing pains, but Mum insisted she was referred to an Orthopaedic Specialist who said she had relaxed muscles. As time went by things didn't get any better, so Nicolina was referred to a specialist at Bath Hospital who diagnosed Morquio disease (MPSIV). By this time, I was born and it was found I had the same condition. Both Nicolina's hips were out of joint so she underwent surgery to put them back in place and was in a frog plaster. She took over the whole back of our car and also a twin buggy – there was no room for me!! After going through all of this, Nicolina's hips slipped out again and it was discovered she didn't have any ball and sockets for the hips to stay in place. She never really walked again.

As we are talking of a long time ago, there was no way Nicolina could cope with attending a secondary school, and as we lived at West Huntspill, Near Highbridge, she went as a weekly boarder to a Barnardos School in Taunton. It was an amazing school and Nicolina took part in some brilliant school plays and went to Stoke Mandeville where she won a gold medal for swimming. At the age of 16 Nicolina went to St Loye's College in Exeter, the first year was Further Education; the second year she did training as a Data Prep Operator and left college with a Diploma.

Both Nicolina and I have had such loving and supportive parents, we grew up believing 'it's not what you can't do, but what you can do'. That saying remained with us always. We learnt to drive and our first car was a little blue mini.

After leaving college, there weren't many jobs around so Nicolina did some voluntary work in an office in Taunton. Later she found a paid job in the Probation Service doing office work. Her final job was at Somerset County Council as a Data Prep Operator and then in the Trading Standards Office which she loved. She made many friends and she worked there for over 25 years.

Three years ago Nicolina had pain in her knee and after trying various treatments she was referred to a specialist at Musgrove Park Hospital where it was diagnosed that she had a Sarcoma tumour in her knee. The only course of action was to have her leg removed, and as there were problems with the anaesthetic due to having Morquio, the only place where this could be carried out was in Oxford – many miles away from her family. A very special friend, Jo, supported Nicolina and us throughout this stage and stayed with her throughout the surgery. The operation was carried out using a light anaesthetic, an epidural and a leg block and within three hours Nicolina was back in her room watching Deal or No Deal!!!! Within three days she was back home with us.

It was on one of her routine three monthly chest scans that it was discovered she had a tumour on her kidney and a smaller one on her lung. She had six sessions of chemo and after the second when her hair started to fall out, she asked a friend to shave her head. She refused to wear wigs and she looked beautiful with her bare head, she was the same Nicolina, my big sister.

“We grew up believing it's not what you can't do, but what you can do”

After all the chemo, the tumours had shrunk slightly but it became clear that the cancer was terminal. It was recommended she try a non-evasive chemo but after just one session Nicolina had a reaction to it and ended up in ITU. We honestly believed we were going to lose her then but with Nicolina's determination she came back home again.

Unfortunately this chemo caused her muscles to weaken and she lost the use of her arms completely. She needed her drink by a straw – even a glass of wine!!!

Every year Nicolina would go to see a different show at the Bristol Hippodrome with friends; it was so sad that this year's trip to a show had to be cancelled.

Nicolina had so many problems but I can honestly hand on heart say, I never heard her say 'why me' or moan. There must have been times when she was in pain, but it was only in the final couple of weeks she would take a painkiller.

On 25th June, much against her will, Nicolina had to go into hospital as she was having difficulty breathing. On 29th June Nicolina passed away.

Our family life will never be the same. Nicolina has left such a big gap both in our hearts and house. It's just Mum, me and Gertie our dog now, but we're sure Nicolina is looking down on us and smiling as she was never a sad person. She will be one of the brightest stars up there.

Sarah Stangoni

Childhood Wood Remembrance Day



The Childhood Wood Remembrance Day is an opportunity for families who have planted a tree in memory of their loved one, to get together.

Starting with a short memorial service, led by Christine Lavery and Trustee Wilma Robins, the families then went off to find their trees and see how much the saplings had grown. A picnic then followed, giving those attending a chance to catch up with each other.

Once the picnic was finished with, everyone then moved on to enjoy the activities Sherwood Pines had to offer - which consisted of the thrilling Go-Ape tree top adventure (not for those afraid of heights!) or a chance to go off-road with forest Segways.

It was safe to say that everyone was able to remember their loved ones and celebrate their life with fun and laughter as well as peaceful recollection.

Rena and Diyen Ramani emailed in to tell us how their day had gone

"We just wanted to say a big thank you for organising a wonderful day to visit the woods. We had a nice day, and means a lot to us that Kayen is remembered. It is also a great way to meet other families as we don't often get to see them. Its just amazing what has been done in the woods and is so peaceful to visit."



A Brief History of Childhood Wood

In 1992 the Society was asked to propose an idea to commemorate children who had died from an MPS or related disease. Out of many ideas, the Childhood Wood was born.

The following year the MPS Society was given an area of Sherwood Forest under licence to create a wood of saplings which were cloned from Sherwood's Major Oak, thought to be between 800 and 1,000 years old.

In February 1993, 150 saplings from the ancient Oak were planted by MPS families, supported by Sir Andrew Buchanan, Lord Lieutenant of Nottinghamshire, along with local MPs and representatives from Nottinghamshire County Council.



The first sapling to be planted was in memory of Simon Lavery who died from Hunter Disease MPS Type II, in 1982.

Since then each of the oak trees planted annually celebrate the life of a child whose childhood was destroyed by these cruel, degenerative diseases. These trees which were planted in the early years are now branching out and forming a canopy.

Over the years further saplings have been planted by families and friends of children who have lost their lives to MPS or related diseases,



The Annual Tree Planting in October

The annual Tree Planting Day takes place during October. Families are invited to attend and plant a tree in memory of their loved one. The day starts with a lunch at a nearby hotel with all the bereaved families and they are joined by local dignitaries, including the Lord Lieutenant and Councillors from Nottinghamshire. After lunch we gather in the Childhood Wood, where the names are read of those children who have lost their lives to MPS or related diseases, and we listen to the beautiful poem 'Remember'. This is followed by the planting of a sapling and the release of balloons by those who have gathered today to remember their children.

Childhood Wood Remembrance Day

Each Summer the MPS Society invites families who have lost sons or daughters to MPS or related diseases for our annual Remembrance Day. Families gather in the tranquillity of the beautiful Childhood Wood to attend the Remembrance for those who have lost their lives; this is then followed by a balloon release in their memory.

Over the years and working in Collaboration with the Forestry Commission the MPS Society has put in place, memory boards and new pathways, all of which are accessible to visitors to the Childhood Wood.

Walking through the pathways, there are a number of wooden animals and a picnic area which are dotted throughout the area, which offer a peaceful setting for those who wish to reflect on their thoughts, before leaving the Childhood Wood.

Events

Legoland Family Day



We love hearing your accounts of our family days and other events, so thank you to everyone who posted on our Facebook page, wrote in or emailed us.

Below is a letter from Justine and an email from Fatima, both of which really sum up the fun and excitement of the day.

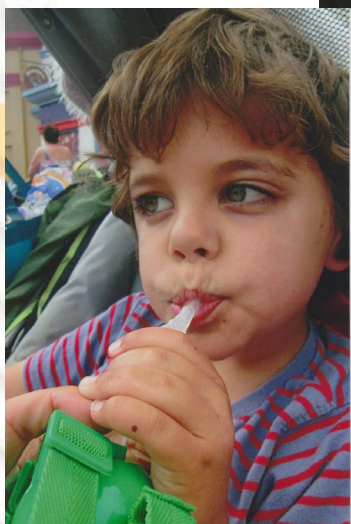
A massive thank you to all who made Legoland possible last Sunday. It was such an amazing day from start to finish.

Freddie very nearly dived into the water on the boat ride to cool down, but was restrained from doing so! He loved the helicopter ride and the train trip. Joseph was in heaven with his re-fillable drinks container, submarine journeys, rollercoasters and pirate ships.

It was amazing to meet other families and not to feel different standing out from the crowd with an overly noisy, excitable Freddie.

It was just the best family day and we are hugely thankful for all who supported it and facilitated it. The boys will remember it for a long time to come.

*Justine De Gennaro
Freddie's mum
(MLIII)*



As soon as I saw the sign for Legoland my sister and I could not contain our excitement! It was a lovely sunny day too.

We met the lovely ladies at the gate who gave us our tickets. We got refillable bottles to fill with soft drinks too. A wasp wanted to share my drink and I kept shooing him off but I think it loved the drink and would just not go away!

We went on the train and we got to see the whole theme park. One of the rides we went on was the Scarab Bouncer ride in Pharaoh city. It was like being stuck mid-air on a magic carpet with butterflies in our stomachs! One of the other fantastic rides was going in a submarine underwater in Atlantis. We really did see a shark... well it was a Zebra shark as the captain announced after a short pause! My sister wanted to go to the adventure playground and see all the miniature buildings like Big Ben and Eiffel Tower and Taj Mahal. It was like visiting the whole world in 1/2 an hour.

After going on a few more rides it was time to go home as my twin baby sisters were really tired.

A big thank you to the MPS Society for organising such a wonderful day out! :-)

*Fatima Narjis
Aged 7*

Our Day at Legoland

by Sally Mitcham

The weeks leading up to the confirmation of Danny's diagnosis with MPSII were probably the most stressful of my life. The knowledge that my sweet, curly-haired little chunk could have a condition with such serious health implications is the sort of prospect that no parent should have to face.

Of course, when our pediatrician mentioned MPS, the first thing I did on getting home was to google it, and pretty soon came across the Society but didn't feel able to get in touch when it was only a possibility at that time. Once we got the positive screening test I plucked up the courage to call and was instantly made to feel welcome and less alone. To know there was an organisation out there ready to offer help and advice made things feel so much more manageable.

Only a few weeks later, after our first session of ERT at Manchester, we got the letter about a trip to Legoland. First thought - it's a really, really long way from York. Second thought - how can we say no to an offer like that, when our eldest is so mad keen on Lego at the moment?

The day itself didn't start well: I hadn't been very impressed with the hotel, the kids had stayed awake hours later than usual the night before, and when we set out to get the car we discovered that our buggy had a flat tyre and we'd forgotten to bring the spares.

The thought of having to carry Danny around the whole day was more than a little depressing - he's a pretty solid three and a half year old!

However, we were greeted at the entrance by friendly faces and a brilliantly organised pack of tickets and so on. I also got to meet Debbie who had very patiently listened to me sobbing at her on that first phone call. Within half an hour we had hired a pushchair (with steering wheel, an instant hit with Danny), got our Ride Access pass sorted and filled up the boys' reusable bottles with their first sugary drink of the day. We were ready to roll!

The highlight for me was the action-packed Pirates of Skeleton Bay show that we happened to come across just as it was starting. As Danny is only at the single word phase, each and every word he says feels quite special still, so I loved hearing him shouting along at the show, and doing his best pirate impression - 'Arrr!' The Dino Safari was a massive hit with him, though he did complain vociferously when he had to get off at the end. The boats and trains in Miniland also caught his interest - it was the quietest place in Legoland so was the best chance we had of getting him to have a nap, but no chance.... If he could speak his only complaint would have been that there was nowhere to play football. Joseph on the other hand was happy as anything - his favourite bit was 'Everything!'

Thanks to a kind grant from the CloverTrust and the Bruce Wake Charitable Trust we were able to take a group of families to Legoland on a sunny Sunday 9th August.

Legoland had something to suit everyone, whether that was a wild water ride or an adventure in Heartlake City. There was so much to see and do that our families left tired but happy. Thank you to the CloverTrust and the Bruce Wake Charitable Trust for funding this wonderful day out.



Being so new to the Society I was hoping to meet some other families, but this is obviously difficult in such a big area. However I did keep an eye out as we were going around. Apologies if anyone noticed me staring at their child - I was just trying to work out if they were another of us! And we did chat to a couple of other families while waiting in queues. It may seem such a little thing, but after months of feeling very isolated, it's good to have found a 'community' where we belong.

Many thanks for the MPS Society for arranging this, and to the kind sponsors who made it possible.



International

Shirley & Sam go to the 4th ISMRD Conference, St Louis, USA

Hi, I'd like to tell you about our trip in July to St Louis, Missouri, USA to attend the 4th ISMRD Glycoproteinoses International Conference.

The conference specialised on the 'ultra orphans' of the Lysosomal Storage disease family, which encompass over 40 similar disorders including the Mucopolipidosis, Alpha – Mannosidosis, Fucosidosis, plus many others.

Sam is 12 and has Mucopolipidosis type III. Sam was diagnosed with MLIII in 2009. Since then, the only information we have had regarding MLIII has come from the various specialists at St Mary's Willink Centre and the MPS Society. To be honest, this was ok as we felt they told us everything we needed to know.

In December 2014, we were lucky enough to attend the MPS conference in Orlando. By chance, we met a young lady and gentleman who were both living with MLIII. We spoke to them and their families and shared many experiences about the diagnosis and how it affected them and their families. We felt so relieved that someone else knew what we had been going through. Many of our experiences were the same. They told us about the conference in St Louis in July 15 and told us that this would be aimed more at ML conditions.

“It was like a sneak peek at the future for Sam”

When we returned home, I joined the ISMRD facebook page. People from all over the world were sharing their support and experiences of ML and asking each other for help and advice. We could identify with a lot of the questions as we were thinking the same things ourselves.

After having just returned from Orlando, we knew we couldn't afford to go to St Louis in July, so I put it to the back of my mind and just kept up to date with the ISMRD sites.

Approximately in February, Sam had a bit of a blip and started rebelling, becoming difficult and blaming his

MLIII for all his behaviours. He started surfing websites for information on MLIII and asking questions and finding excuses. With a heavy heart, I asked his professionals for help and advice. Sam's OT said we should apply for funding to try and help us attend the conference. Luckily, we were awarded a large donation and we fundraised for the remaining balance. So in July, off we went to the conference in St Louis.

There were approximately 20 people present who are affected by MLII/III and MLIII from USA, Canada, Australia, New Zealand, Norway, Ireland and England. It was lovely to meet Sarah Burgess from Luton who also has MLIII and Aoibhe O'Connor (MLII/III) and her family from Ireland. We didn't feel as much alone from a tiny island in the North Sea.

The trip and conference were a success to both Sam and myself as it gave us a chance to speak to others who are affected by Mucopolipidosis, share our life experiences and generally just let Sam and the others be themselves. The age ranges were between 4 to 40 years of age. Sam made friends with mainly the lads/men and boys of course. They compared wheelchairs, whose was the fastest? Whether they could make a fist, or put their knees together? Who had had the most operations and who had recovered in the quickest time! They also just talked about sports, sweets and music like most people do.

The lads I expected Sam to 'hang out' with, were, when I thought about it, young men in their 20's, and what would they have in common with Sam? They just still looked youthful, but they were lovely guys who were setting a positive example to Sam in that they were doing their best to remain mobile, still studying at college, and wanting the same things in life as their friends and siblings. I suppose it was like a sneak peek at the future for Sam. In the main, Sam played with kids his age, generally causing havoc playing indoor tennis and squashing balloons.



I gained a lot from speaking with the families of those affected. Many of them had met previously but were very supportive and friendly towards the new-comers. At times I felt rude asking lots of questions, but I suppose they knew I needed answers and they could tell me from their experiences.

The speakers at the seminars were also very down to earth and approachable. I don't confess to understanding everything they were discussing, but it was all relevant to ML and in some cases supported my sketchy previous knowledge.

Sam enjoyed the whole experience and enjoyed making new friends who I'm sure he'll meet up/contact again in years to come. There was only one little blip where he asked me if he was going to end up in a wheelchair all the time. I said there may be a possibility, but if he let us help him make the right decisions, we would help him to stay mobile for as long as we could. His little face creased up and tears came rolling down, but within 30 seconds, he pulled himself together and carried on eating as if it didn't apply to him.

To conclude, the ISMRD conference was a success for us both in giving us a bit more confidence and knowledge about the condition. We no longer feel isolated or alone as we know we have friends all over the world who know what we're going through. We can contact them anytime if we need to rant, ask advice, or give support.

*Shirley Jamil
Sam's mum*

Photo shows Sam and Sarah Burgess

Research & Treatment

How the MPS Society works alongside the NHS on behalf of MPS and Fabry patients

Fabry, MPS and related disease patients in England are managed at one of eight centres of excellence. The MPS Society works closely with all of the centres and attends the regular Expert Advisory Group (EAG) meetings chaired by Edmund Jessop, of the Specialised Commissioning Team NHS England to support the development of the service and to ensure that the patient voice is always heard.

These meetings, held two or three times a year, bring together physicians from all eight centres and members of other patient organisations from various LSD charities. The focus of the meetings is to discuss the management of the LSD Service, the treatment options, research, treatment protocols and future service developments. The report below covers the work that has been done over the past six months.

NHS England consultation on how Specialised Services are commissioned

In England the NHS is responsible for commissioning treatments for patients. Under new arrangements which came into force in April 2013, in order for NHS England to pay for new technologies (which are not subject to NICE) there are a number of different pathways and committees to go through. Finally, a group called CPAG make a final decision before it is rubber stamped for financial approval and then made available to patients.

In a recent meeting with James Palmer and Richard Jeavons the MPS Society have learnt that all new medicines for ultra rare diseases will go through a NICE Highly Specialised Technology Appraisal and there will not be 'Interim funding' by NHS England to bridge the gap between a medicine receiving marketing approval by the European Medicines Agency and the completion and decision of the NICE appraisal.

Patient/Charter Agreements

Members of the UK LSD Patient Collaborative will be working with NHS England over the coming months to develop these agreements which will be issued to all new and existing patients. It will explain what a patient can expect from the treating centre and the patient's responsibility in

return for receiving clinical management and treatment of their condition. A letter will be sent to all patients prior to their hospital appointment informing them they will be issued with this agreement enabling them to bring along representation/support if they wish.

Homecare Contract

Patients with MPS or Fabry disease who receives ERT are supported by a national homecare service, allowing them to receive their infusions at home. The service enables patients to become independent infusers through training or to be semi or fully supported by a trained paediatric or adult nurse. This is managed through each of the eight hospital trusts who work with the appointed homecare company to meet the needs of their patient cohort.

The first contract (2012-2015) attracted four homecare companies into the framework; one company – Medco, withdrew in 2013 and another – Central - were unsuccessful in recruiting patients into their service. This left two – Healthcare at Homecare and BUPA homecare - to service the needs of LSD patients throughout England. The current contract ends in October 2015 and over the last year representatives from each of the eight treatment centres and Tanya Colin-Histed, Chief Executive of Gaucher Association, as a representative of the UK LSD Patient Collaborative have been working alongside staff from the NHS's Commercial Medicines Unit to develop a new contract to take the service forward. It is anticipated that a number of new companies may tender to be part of the LSD Homecare contract which will drive up quality and choice for patients.

The contract will describe the level of service that patients should receive from their homecare company and set out guidelines and working practises that the companies should adhere to that ensures safety and quality to patients.

*Christine Lavery
Group Chief Executive*

FDNA project - Help Geneticists identify MPS patients earlier

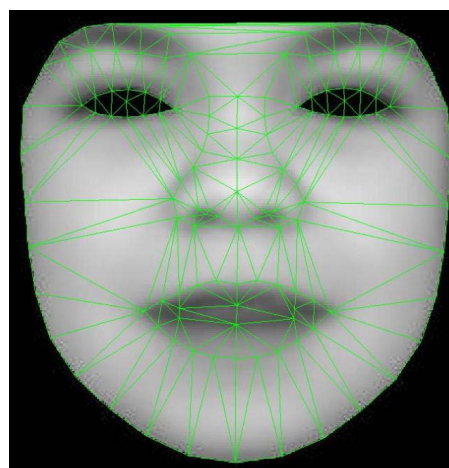
We are supporting a project in mucopolysaccharidosis (MPS) patients working with a company called FDNA (Facial dysmorphology novel analysis). As you may be aware, many genetic diseases are associated with the gradual development of distinctive facial characteristics in affected patients. FDNA have developed specialized software that analyses facial images with the aim of being able to use this facial image analysis to identify facial morphology associated with rare diseases and to be able to monitor disease progression.

The software developed by FDNA converts facial images into a mathematical algorithm that describes the characteristics of each facial feature and the spatial relationship of facial features eg. width of the bridge of the nose, thickness of the lips, distance between the nose and the corners of the eyes and distance from the tip of the nose to the corners of the mouth. FDNA are collecting data from images of patients with many different genetic diseases and have developed their software to be able to successfully differentiate patients with a number of rare diseases. FDNA do not retain any of the children's images, the photos will be scanned and the only information retained is the numerical information, so all the data is automatically anonymised.

For MPS the FDNA software is currently able to distinguish patients with MPS from normal patients and from patients with other non-MPS syndromes. The aim of this current project is to see whether they can develop the software further to be able to distinguish between patients with different types of MPS eg. MPSI from MPSIII, or MPSIIIA from MPSIIIB. To be able to do this they need to collect data from as many facial images as possible from children at different ages with different types of MPS. Differences between some MPS subtypes are very subtle and may not be discernable by eye, so this will test how sensitive the system can be.

We invite you to participate in this project and upload photographs of children with MPS into this secure portal:

<https://community.FDNA.com/MPSUK>
Access code: **MPSUK2015**



Serial photographs of the same child at different ages are particularly useful for showing changes as the disease progresses, so if possible please upload past school photograph type photos of your child at different ages. Thank you for taking the time to read about this project. There is no obligation for you to take part if you do not wish to.

If you require help uploading to the secure portal, please contact Emma on 0345 389 9901 or e.henry@mpssociety.org.uk

Thank you to all those who have already contributed to this important project.

FDNA's technology automatically extracts de-identified binary information from the photographs uploaded to perform the analysis. As more information is collected, the technology learns and becomes more accurate for the benefit of the entire genetic expert community and their patients.

save the date

14-17 July 2016, Bonn, Germany

Satellite Meeting:
13. - 14. July
- Biology of the Lysosomal Network

MPS 2016
14th INTERNATIONAL SYMPOSIUM
ON MPS AND RELATED DISEASES

European Commission Grants Marketing Authorization for Kanuma™ (sebelipase alfa) for the Treatment of Patients of All Ages with Lysosomal Acid Lipase Deficiency (LAL-D)

CHESHIRE, Conn.--(BUSINESS WIRE)--Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced on 1st September that the European Commission (EC) has approved Kanuma™ (sebelipase alfa) for long-term enzyme replacement therapy (ERT) in patients of all ages with lysosomal acid lipase deficiency (LAL-D). Kanuma, an innovative ERT, is the first approved treatment in the European Union for patients with LAL-D, a genetic and progressive ultra-rare metabolic disease in which patients suffer multi-organ damage and premature death. Alexion expects to begin serving patients in Germany in October and is now commencing reimbursement processes with healthcare authorities in each of the major European countries.

“Today’s approval is a crucial milestone for patients with LAL-D, a grave condition that can have devastating consequences for patients of all ages,” said Vassili Valayannopoulos, M.D., Ph.D., investigator in the Kanuma pivotal studies, Hôpital Necker-Enfants Malades and IMAGINE Institute, Paris. “In clinical studies, 67% of infants treated with Kanuma survived beyond 12 months of age, whereas without treatment, these patients would have faced a near-certain fatal outcome. In pediatric and adult patients, Kanuma was also shown to reduce the markers of liver injury and lipid accumulation, which can lead to serious and life-threatening complications.”

LAL-D is a genetic, chronic and progressive metabolic disease in which infants, children and adults suffer multi-organ damage and premature death. It is an ultra-rare disease, which is defined as a disease that affects fewer than 20 patients per one million of the general population.¹ Patients with LAL-D often experience a rapid onset of life-threatening disease manifestations, and similar to other liver diseases, many patients may be asymptomatic until they experience

a severe consequence of the disease. LAL-D is caused by genetic mutations that result in a marked decrease or loss in LAL enzyme activity in the lysosomes across multiple body tissues, leading to the chronic build-up of cholesteryl esters and triglycerides in the liver, blood vessel walls and other tissues.^{2,3}

“We are pleased that the European Commission has approved Kanuma for patients of all ages with LAL-D, enabling us to serve infants, children and adults in Europe with the first approved treatment for this ultra-rare, severe and life-threatening disease,” said David Hallal, Chief Executive Officer of Alexion. “In the absence of any effective therapy, patients with LAL-D face devastating morbidities including liver failure and premature mortality. We are grateful to the investigators, patients, and their families who participated in the clinical trials that made this approval possible and we are now commencing reimbursement processes with healthcare authorities throughout Europe to ensure that patients with LAL-D have access to Kanuma, a life-transforming treatment, as quickly as possible.”

Kanuma is a highly innovative enzyme replacement therapy (ERT) designed to address the underlying cause of LAL-D. The approval of Kanuma applies to all 28 EU member states as well as Iceland, Norway, and Lichtenstein and was granted under the accelerated assessment procedure. The decision follows the June 2015 positive opinion granted by the Committee for Medicinal Products for Human Use (CHMP). In addition, the U.S. Food and Drug Administration granted Breakthrough Therapy designation for Kanuma for LAL Deficiency presenting in infants and accepted the Kanuma BLA (Biologics License Application) for Priority Review.

Longitudinal Study on Bone Disease in MPSI, II and VI

University of Minnesota Study

Dr Lynda Polgreen, a paediatric endocrinologist at the University of Minnesota, is conducting a study on bone growth and bone health in children and adults with MPSI, II and VI. The aim of the study is to better understand the skeletal disease in MPS and to determine the progression of skeletal disease in MPS which is important for future clinical trial outcomes.

This study is taking place at three US sites; Oakland, California; Salt Lake City, Utah and Minneapolis, Minnesota. For further information please contact Dr Polgreen polgr001@umn.edu or study coordinator, Jane Kennedy kenne634@umn.edu

Managing Your Mental Health as a Metabolic Conditions Patient

by Rebecca Southall & Alan Naughton
Specialist Psychological Therapists
Prohealth UK

In a report investigating the emotional impact of rare life-long health conditions in the UK, 53% of those surveyed felt they had no one to turn to in the medical system for information or support, 69% had feelings of depression, 82% had feelings of stress or anxiety, and 91% worried about how their health might change in the future. (Shire, April 2013)



For many people, lifelong illness can be both physically disabling and emotionally exhausting to manage. Some people may not have access to or respond to available medical treatments which can add to the sense of frustration. In some cases people are told by healthcare professionals on diagnosis...

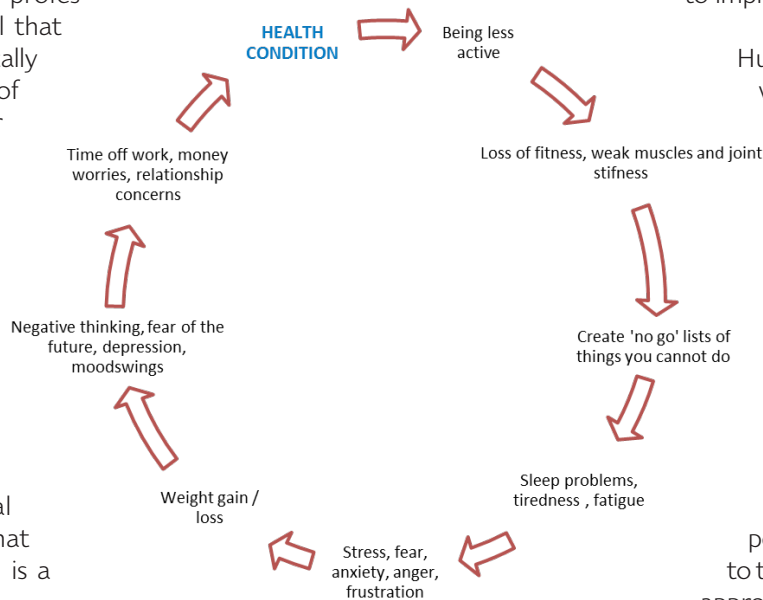
"I'm afraid you have a life-long health problem. You will have to learn to deal with it".

To date, your healthcare professionals may have done all that they can to help you physically and may have used many of the tools available in their toolkit. However, there are so many things that you can do to help self-manage your emotional health alongside the support of your health care professional, family, friends and work colleagues. First though you need to be willing to ask for information and emotional support, so knowing what is out there to assist you is a great place to start.

Local Services

You may also wish to seek one-to-one support in a therapeutic setting. This can be provided through your local Improving Access to Psychological Therapies (IAPT) service to which your GP will be able to refer you. Alternatively, there are many private counsellors and therapists that can be found on the websites of professional counselling bodies such as the BACP and the BABCP.

There are various different therapeutic approaches available. CBT works by helping you make sense of overwhelming problems by breaking them down into smaller parts. Your thoughts, feelings, physical sensations and actions are interconnected often trapping you in a negative spiral. CBT helps you stop these negative cycles. It aims to break down factors which are making you feel bad, anxious or scared so that they are more manageable, and can show you how to change these negative patterns to improve the way you feel.



Humanistic therapy takes a whole-person approach to your problem, using a range of theories and practices to help you develop self-awareness and autonomy, these therapies explore your relationship with different parts of yourself (such as your body, mind, emotions, behaviour and spirituality) and other people (for example family, friends, society or culture) whilst supporting you to grow and live life to the full. Variations of humanistic approaches include Person Centred, Gestalt and Transactional Analysis.

Psychodynamic therapy aims to help individuals to unravel, experience and understand their true, deep-rooted feelings in order to resolve them. It takes the view that our unconscious holds onto painful feelings and memories, which are too difficult for the conscious mind to process. In order to ensure these memories and experiences do not surface, many people will develop defences, such as denial and projections. According to psychodynamic therapy, these defences will often do more harm than good.

Mindfulness

Mindfulness is a form of meditation that originated in Buddhism 2,500 years ago. It is the act of paying attention in a particular way and involves bringing your attention back to the present moment, both purposefully and non-judgmentally.

The aim is to become aware of the full range of experience including sensory impressions, thoughts, imagery, emotions, urges and impulses. We even can become aware of the quality of our mindfulness itself – whether our mind is clear and organised, or jumbled and disorderly. Because we do not judge either the content or the processes of our mind, we become freer to observe our environment without identifying with the contents of our thoughts. The purpose of mindfulness is not change, but acceptance, non-judgment, patience, curiosity and letting go.

Mindfulness techniques are particularly effective for those people with lifelong health conditions, as they have been shown to improve both your physical and mental health. A reduction of pain, better quality sleep, lower anxiety and improved mood are all benefits of practicing mindfulness.

Space4U Project

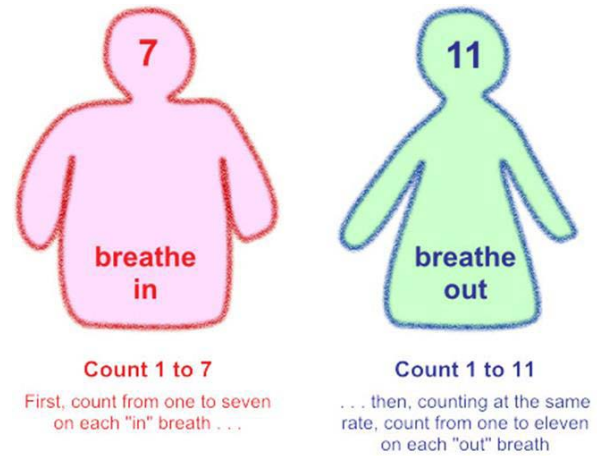
You can find a free downloadable Mindfulness Handbook which contains a variety of exercises and techniques for different learning styles on our Space4U2Talk website at www.space4u2talk.com/mindfulness

Our Space4U2Talk website provides online support and resources for a number of our ongoing projects, including the Long Term Conditions Service, Post-Natal Wellbeing Service, and Peer Wellbeing Champion training. Here you can find free Wellbeing resources all downloadable in pdf format.

6 Ways to Wellbeing

Our '6 Ways to Wellbeing' are based on an extensive review of the actions people can take that are positively associated with mental health and wellbeing. They describe some of the key components of a healthy emotional or social 'diet'. All 6 of these suggestions are free, easily achievable and applicable to anyone's life regardless of their circumstances. Many of us will be already doing some of the '6 ways' without even being aware of it, but when we decide to change our behaviour or learn new skills, we need to put them into practice and make conscious attempts to keep doing them regularly. Making anything part of our life needs commitment and perseverance.

To find out more about the '6 ways to Wellbeing', visit www.space4u2talk.com/wellbeing, where you can download a free wellbeing handbook that contains information, advice and a reflective journal so you can keep track of your progress.



Increasing your capacity for mindfulness supports many attitudes that contribute to a satisfying life. Being mindful makes it easier to notice the pleasures in life as they occur, helps you become fully engaged in activities, and creates a greater capacity to deal with difficult events. By focusing on the here and now, many people who practice mindfulness find that they are less likely to get caught up in worries about the future or regrets over the past, are less preoccupied with concerns about success and self-esteem, and are better able to form deep connections with others.





New Drug Driving Legislation Alert

New drug driving legislation came into force on 2 March 2015. The Drug Driving (Specified Limits) (England and Wales) Regulations 2014 introduce a new offence of driving, attempting to drive or being in charge of a motor vehicle on a road or other public place with amounts of any of 16 controlled drugs above specified limits in the driver's blood.

What is changing?

Previous "drug driving" legislation makes it an offence for a person to drive or attempt to drive a vehicle if they are unfit to drive through drugs. For a successful prosecution, it needs to be proved that the driver's impairment was caused by the drugs in their system. There have been no objective standards by which to determine this, which has attracted criticism due to the relatively low numbers of prosecutions for this offence to date.

This law will remain in force, but the key impact of the new legislation is that if a driver is found to have amounts of one of the specified drugs above the prescribed limits, they can be convicted on that basis, without any evidence of impairment and without there needing to be a link between any impairment and drug use.

As might be expected, the drugs listed in the legislation include illegal drugs such as cocaine. However, they also include drugs included in medicines which are either legally prescribed or available over the counter such as lorazepam.

Specified drugs include:

- Benzoyllecgonine
- Lorazepam
- Clonazepam
- Lysergic Acid Diethylamide
- Cocaine
- Methadone
- Delta-9-Tetrahydrocannabinol
- Methylamphetamine
- Diazepam
- Methylenedioxymethamphetamine
- Flunitrazepam
- 6-Monoacetylmorphine
- Ketamine
- Morphine

What is the potential impact of the new offence?

The Department of Transport (DfT) estimates that around 19 million prescriptions a year are issued for drugs which are included in the scope of this offence. This means that drivers are at risk of inadvertently breaking the law when taking certain commonly used medicines.

Police forces have been issued with mobile screening devices, and any roadside testing would be followed up by blood and urine tests at a police station. However, these screening devices can only detect cannabis and cocaine, and concerns have been raised about their accuracy.

The DfT has reassured drivers that people who use the drugs listed legitimately, following the advice of a healthcare professional will be able to drive without fear of being prosecuted. There will be a defence for drivers who were prescribed the specific drug for medical or dental purposes and took it as directed in accordance with the instructions (insofar as consistent with the directions from the healthcare professional). However, this defence will not apply if the driver ignored any advice given when prescribing or in the instructions about the amount of time that should lapse between taking the drug and driving. Despite the DfT's assurances, this has raised concerns about whether the defence would apply in circumstances where a patient is prescribed an off-label unlicensed medicine.

Drivers have been advised to keep evidence of any legally prescribed medicines with them to speed up the investigation process should they be stopped, but this will be particularly important for off-label or unlicensed use. The consequences of getting this wrong can be stark, involving up to six months' imprisonment, a fine of up to £5,000 and a minimum of 12 months' disqualification.

What we are doing?

According to press reports, almost half of UK drivers are unaware that the new legislation is coming into force. This means that charities play a key role in raising awareness of these changes. We are raising awareness amongst our members as some of you may be taking medication or using drugs on the list above.



Learning Support Assistant Rachel raised an astounding £2,586.25 in support of Sophie & Tom (MPSIII).

Read her story on page 27

Fundraising

Welcome to the fundraising section of our Autumn 2015 MPS Magazine.

As you may have seen, we are again holding our annual National Draw to raise money to support those affected by MPS, Fabry and related diseases. We hope that you all get involved and sell as many tickets as you can. You can read more about the National Draw on page 37.

We have seen some fantastic fundraisers over the summer, including parties, music events, runs, football matches and cycles. If you would like to recreate any of the fundraisers you see in this section, get in touch with us for advice or to request a fundraising pack - just email the fundraising team on fundraising@mpssociety.org.uk or call 0345 389 9901.

For those of you who welcome a challenge, why not add

your name to our list for some of our running events for 2016? The British 10K London Run, the Great North Run, the Great South Run and the Great Manchester Run are some of the events we will be hoping to obtain charity places for.

If you are on the list for one of our very limited places for the 2016 London Marathon, you will be hearing from us at the end of October. Good luck!

If you have a fundraiser planned, don't forget to let us know how it went - your photos and articles might even end up in our next magazine!

Many thanks for your support.

Elkie Riches
Fundraising & Information Officer



Rachel's Raise & Save

I first met Sophie & Tom in September 2013 when I got a job working at their school as a Learning Support Assistant. Then in April 2014 I began working with them at home every evening and some weekends/school holidays. Sophie is 12 years old and Tom is 10, and they were diagnosed with Sanfilippo in 2006 aged 3 & 1. Working with them every day I have fallen in love with them. Spending every day with them wasn't enough because although I can play with them in the garden or feed them or bath them, I can't save them. So in January this year I got to planning the fundraiser. And once I got that ahead, my Dad began on the football tournament.

On the 3rd June my Dad and his work friends organised a charity football tournament with all money raised going to the MPS Society. 17 teams took part in the event from the Heathrow area, with the first three placed teams receiving medals and the winners awarded a cup, all donated by my Dad's company, Atkins. They raised an amazing £1000!

Then on the 26th June I organised the 'Raise & Save for Sanfilippo' Night at Totton & Eling Cricket Club. The club is kind enough to hire the huge hall and outside area for free for charities. We had various things running on the night, as listed below that were all donated or hired out for a discounted price:

- A raffle with over 25 prizes donated by local businesses including family tickets to Paultons Park & specialist treatments at Sophia's House (a therapy clinic for Special Needs children)
- Four large sumo suits
- A secret auction with 3 homemade Disney costumes,
- A signed photo of Jenson Button & two England Rugby shirts
- A photographer, Laura White, who set up a photo booth where we dressed up and she took fun photos for us
- Olaf & a minion came to the party for the evening to take pictures and dance with the children
- A cake sale, and a two tiered cake donated by the head chef at Sophie & Tom's school to auction
- Live music from Tom Stride & his guitar, followed by a DJ & Disco and a game of Disney Rock & Roll Bingo
- Totton & Eling Cricket Club provided us with a bar & BBQ

We had an amazing turn out with over 100 people coming to the night for support, including Sophie & Tom and their family. The night raised £1450.40!

All together we raised an amazing £2,586.25!

Rachel Healy

Charity of the Year

Help us continue to provide support, fund research and raise awareness...

Make The MPS Society your company's Charity of The Year!

A Charity of the Year Partnership scheme can demonstrate your company's commitment to corporate social responsibility, whilst committing to more sustainable support for your chosen charity. It provides opportunities to generate positive publicity with a clear message that can strengthen brand image, reputation and awareness. The scheme can also be a successful tool for building team spirit and boosting staff morale, bringing employees together in an organised, coordinated way. We have plenty of ideas for how to make partnerships successful and memorable and are dedicated to engaging and motivating staff. Adopt us as your Charity of the Year (COTY) and your staff can play a vital part in our work.

In memory of



Chloe Walker

We were so touched by this letter that we decided to include it in our magazine.

Leona, Kayleigh and Jess did a fantastic job of celebrating Chloe and Sharon's lives, and we would like to thank them and Chloe's family and friends for so kindly donating to us and raising a spectacular £600.

What an amazing way to remember your friends.

8 September 2015

Dear All at MPS Society

Our friend Chloe passed away on May 24th 2010 on her 16th birthday when the bus she was travelling home from Keswick School on, was involved in a collision.

Chloe's brother Jordan died in 2007 from the degenerative condition, sanfilippo, which was diagnosed when he was seven years-old.

Last year her mum, Sharon, unfortunately passed away after a short battle with cancer.

This year should have been Chloe's 21st and although she couldn't be there we had a party to celebrate her and her mum's life and to raise money!

We have enclosed a cheque for £600 which was raised on the night by all Chloe's family and friends.

We cannot thank you enough for the work that you do so hopefully this will show some of our appreciation.

Sending Love and Thanks,

Leona, Kayleigh & Jess

Proud friends of Chloe & Sharon Walker





Towersey Morris

Staff from MPS House were recently delighted to be given the opportunity to attend a traditional dance by the Towersey Morris, which was then followed by a cheque presentation. Towersey Morris have given the MPS Society fantastic support over the years, and raised thousands of pounds, so thank you so much to everyone involved - we had a great time!

Below, Andy Hardy explains the connection between Towersey Morris and the MPS Society.

Twenty years ago my son, Matthew, died at the age of thirteen. He had suffered from Hunter's Syndrome. Like many MPS children he loved music and movement – his favourite television programmes were Songs of Praise and Top of the Pops. Matthew went to a lovely special school in Aylesbury and benefitted from having Val Merry as his own PA! Val's husband, Tony, was Squire of Towersey Morris dancers and he suggested that the side 'adopt' MPS as their chosen charity. This they did with relish! Matthew was made an honorary member and had his own waistcoat and set of bells.

The side also tried to persuade me to join them, but teaching and schools' football commitments along with being a MPS parent prevented me from so doing. However, following his death I felt that I couldn't make any more excuses and so, in the summer of 1995 I tentatively began to learn the rudiments of 'one hop, two hop, feet together, jump.' Since those early steps I haven't looked back. Trips to Belgium, Brittany, Lithuania and Macclesfield (!) and performances at the Millennium Dome, on Misomer Murders, in Pudsey the Dog (The Movie) and at the occasional MPS Annual Conference have been just some of the high-lights.

Throughout all this time Towersey Morris have continued to support The MPS Society, and more recently we have also supported The Multiple Sclerosis Trust, as the afore mentioned Val is battling against the disease.

On Wednesday, 29th July 2015 Towersey Morris, along with its 'sister' side, The Three Horseshoes, performed outside the local pub in Towersey. Towards the end of the evening we were delighted to present cheques for six hundred pounds to three reps from each of the two charities. We even got them dancing! The money had been raised from collections taken during our summer season and over last Christmas (when we performed an extraordinary piece of drama known as the Towersey Mummies' Play), adding to the thousands of pounds we have raised so far for both charities.

Next year Towersey Morris celebrates its Golden Jubilee and I hope that we will continue to support The MPS Society long into the next fifty years.



Andy Hardy
Towersey Morris

Back In Action

www.backinaction.co.uk



Back in Action was set up in 1988 to help people manage, prevent and treat the symptoms of back pain. Their four great stores in London, Amersham, Bristol and Marlow have an incredible range of stylish, therapeutic furniture that will compliment any home or office.

Back in Action have kindly donated a fantastic prize to The MPS Society's National Draw, but they have also very generously provided 1 free session on their Mobiliser System to anyone who mentions the MPS Society in any of their four stores.

The Mobiliser is a powerful joint mobilisation system and you can find out more information on this system on their website www.backinaction.co.uk or at any of their stores.

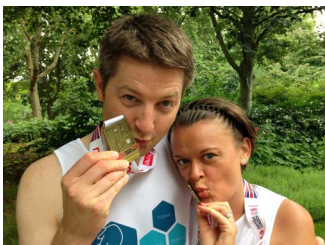


British 10K London Run

On Sunday 12th July six of our supporters completed the Vitality British 10K London run for the MPS Society, an raised an amazing total of £2,647.50!

A huge thank you to Danielle and Tim (photos) and to Emma, Hannah, Daniel and Rachel, who ran in support of Tillie Mae (MPSIII). We hope you had a great day!

Danielle and Tim got in contact with us to let us know that their JustGiving page was one of the most successful page for the month of July: out of 64,492 fundraisers, their page was in the top 3%, which is incredible! Danielle emailed to say "We are so proud of raising so much for this much deserved charity, the email from just giving makes us even prouder of our kind friends and family who have supported us."



If you are interested in taking one of our charity places for the British 10K London Run 2016, please drop us an email to fundraising@mpssociety.org.uk. Places for the Great North, South and Manchester Runs will also be available.



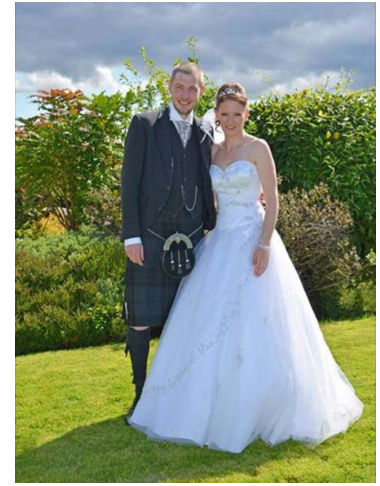
A Night for Jack

On January 24th this year, our amazing community of Calne, along with friends and family came together to celebrate Jack's life and raise money for the MPS Society.

"A Night for Jack" was organised by his friends Benji and Mark as one of their "Damp Sessions" which they decided to dedicate to him.

Jack's friend Robbo then became involved by organising a fantastic raffle, with great prizes. The event was held in the King's Arms, (Jack's local) and was an evening of brilliant music by incredibly talented musicians, singers and DJ's. Jack would have loved it! In total £2,329.95 was raised and a lot of glasses were raised to Jack!

Manda Stuart



Jessica & Neil's Wedding

Neil Cullen married Jessica McArdle on 7 August 2015 at The Beardmore Hotel, Clydebank, Dumbartonshire and in Jessica's own words Its now official "I am a Cullen."

They had a fantastic day with their friends and family and the sun shone all day for them which is rare in Scotland.



Jessica and Neil asked their guest for donations to be given to MPS Society rather than receive wedding presents as their daughter Paige, now 8 years old, was born with Morquio Type IVA.

Paige and her little 1 year old sister Renae were both flower girls at the wedding. On the day the sum of £480.00 was raised, with some of the other guests donating directly.

Catherine Cullen



A Ride for Mucopolysaccharide by Jessica Bumby

Picture this – sitting down all weekend and eating guilt-free cake, while raising money for a great cause. Sound too good to be true? Well that's exactly what we did, with one slight catch, we cycled 127 miles in the process! Over the May bank holiday weekend, myself and 7 other cyclists hopped on our bikes and rode from Leeds to Liverpool along the canal to raise money for The MPS Society.

Day one was fine and dry, we took it steady riding 28 miles to Skipton. It wasn't all cake and sunshine though, as we had the not-so-easy task of cycling up Bingley Five Rise Locks – the steepest flight of locks in the country. Having previously struggled in training to make it to the top without stopping, I set about conquering my Everest! Halfway up with legs of jelly, I recalled a cycling tip that I'd read during sleepless nights of Googling 'how to cycle steep hills without stopping' and decided to 'traverse it'. 'Traversing it' quickly turned into 'cursing it', but I clumsily zig zagged my way to the top. The other riders breezed up far less dramatically, apart from Paul who had his first of many punctures (we soon concluded he'd have been better opting for a penny farthing than the ancient relic he had decided to use!)

Skipton was a welcome sight after navigating the rocky terrain in the miles leading up to it. We settled in for a lovely meal, but in the back of our minds we knew that we had to do it all again the next day...doubled.

Day two was 51 miles to Chorley and it was scorching weather (lucky us) apart from the fact we had forgotten sun cream (not-so lucky us). After beautiful views, swarms of flies and one fall, we met our match – a swan who we lovingly named 'Swanzilla'. Taking up the entire canal path, feathers ruffled, beak wide open and muscles flexing, Swanzilla caused us to dismount our bikes and run past him, as he tried his best to have a lunch of ankles and bike wheels – but we lived to tell the tale! After a good 8 hours of cycling, we were relieved to see the hotel and a sunlit beer garden waiting for us at the end of the day.

The last day brought with it lashings of wind and rain, as we embarked on a very cold, wet, muddy and sore 48 miles to Liverpool. With mud splashes reaching up to our helmets and

soreness that no amount of padded Lycra could prevent, we powered through the final leg of boggy ground, tree roots, rutty paths and diversions to the finish line. The sun came out for the finale, as we were welcomed in by our amazing supporters – Shelley, Mandy and Maisie, who had carted our bags from hotel to hotel and cheered us on every wheel turn of the way.

All in all, the challenge certainly lived up to its name, but was an unforgettable experience and brought an incredible sense of achievement. The best part? We raised £1,462 for The MPS Society, plus, thanks to Paul and Vodafone matched funding, an additional £350 went directly to MPS – so £1,812 in total! A huge thank you goes out to everyone who supported and donated. A special thank you also goes to the cyclists – Darol, Lynn, Murray, Darren, Paul, Michael and Sharon, who threw themselves into the fundraising (and luckily not into the canal) all for a fantastic charity.

(Watch our journey on YouTube – 'Go Pro Leeds-Liverpool MPS Cycle')

LET ME TELL YOU A BIT ABOUT MPS
 it's a rare disease which gets little press,
 AWARENESS and MONEY we're hoping to raise
 as it affects **ONE BABY BORN EVERY EIGHT DAYS.**
 It's genetic, progressive, there's no cure (yet)
 we want to help change this through miles of sweat!

On May 22nd we'll hop on our bikes
 and cycle for **127 MILES** (oh yikes!)
 We've got up for Spin, come sunshine or showers,
 we've practised the route for hours and hours...

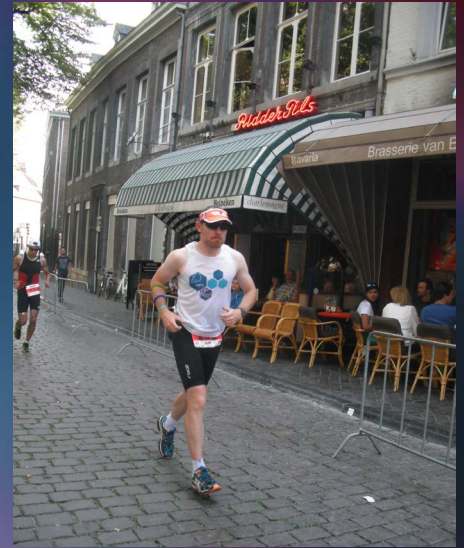
We've got padded pants (ever so flattering!)
 we've fallen in ditches, rain and mud splattering.
 We've trained really hard, the best we can do,
 so now the important bit comes down to **YOU!**

MUCOPOLYSACCHARIDE

PLEASE TEXT MPSS89 FOLLOWED BY £1 (OR MORE)
 to 70070 and we'll guarantee for sure
 That we'll be **REALLY GRATEFUL**, we'll cycle like mad,
 because we know MPS will be ever so glad!

OR VISIT THE WEBSITE BELOW
<https://www.justgiving.com/LtoL>

Above: Jessica's inspired fundraising idea of writing a poem to communicate what she was doing and why - a fantastic way to get your message across and one which helped her to raise an amazing amount of money. Well done, Jessica and everyone involved, and thank you for your support!



My Ironman Journey

2nd August 2015 - Maastricht, The Netherlands

My Ironman Journey began 16 weeks before the race, not long after I had ran the Bath half marathon. Chatting with my wife, Suzy, I considered what things we would like to do this year and an ironman triathlon was at the top of my list. A friend of mine had completed one the year before and I felt that this year was right to 'give it a go'. The race chosen was set in the idyllic and historic setting of Maastricht, The Netherlands on the 2nd of August.

My reasons for raising funds for the wonderful MPS Society is that a large portion of my wife's family suffer from a very rare genetic disorder known as Fabry Disease. Her mother, two aunts, two sisters, five cousins and nephew all suffer from this condition, which has been a tremendous incentive to me to do something to aid their future.

Being a secondary English teacher it was always going to be difficult to find time to train whilst remaining an effective teacher and a present husband. This involved cycling the 35 miles home from school on a regular basis, utilising the pool at school for early morning sessions and then running when I could find time - as my starting point. This quickly increased to cramming in two 1.5 mile swims in a week, at least 15 running miles and

as many miles on the bike as possible on the weekend and weeknights. The event required me to complete, in order, a 2.4 mile swim; 112 mile bike ride and 26.2 mile run, so I needed to be averaging around 10 hours a week in training to give myself a chance.

We arrived into Maastricht on the Friday evening before the Sunday event, continuing the pasta intake as best I could. The excitement and electric atmosphere of the place was palpable all day on Saturday around the registration tent and in the transition area where I racked up my bike. Finely tuned athletes roaming around, with chiselled calves and aerodynamic gear coming out of their ears whilst they assessed the competition, reminded me of what lay in store the following day.

Leaving the hotel at 5:40am we made our way to the transition area to check my bags in and make final checks on the bike etc. The mass swim start took us down the main river of the city, under the government buildings and then back against the tide towards the fans. My nerves thankfully ceased the moment I felt the unusually lukewarm water, allowing me to focus on what I needed to do. Despite a slight bottle neck and occasional elbow I finished part one unscathed. A quick change in

the tent out of the wetsuit and into my cycle gear and I was successfully off on the long bike leg. My fears soon resurfaced as I had a large blow-out of my rear inner tube after only 30 minutes of cycling. My race is over! Being my initial reaction as I only had the aid of a small hand pump; however, a Dutch lady, now a friend, rushed to my aid by bringing out a full cycle pump with the correct size of valve to get me back up and out after only 15 minutes of stalling. Needless to say when I finally got off the bike after 6 more hours of cycling I was relieved and knew that I would finish.

I dragged my body around the three looped run course to finish in 12 and a half hours, concluding with tears and hugs with my patient wife, Suzy, who kept me going throughout the training and especially the race. I would like to thank all that supported me, but most importantly wanted to acknowledge all of the sufferers of genetic disorders related to the MPS and say the pain was worth it to know I have helped even a little of your daily struggles.

Jeff Elliott

Jeff raised an amazing £1,768.26 on his JustGiving page - thank you, Jeff, and congratulations on your achievement!



Women's Glasgow 10K

29th March 2001 and we have just received the news that our youngest son has an MPS disease called Hurler Syndrome and that we were more than likely to lose him in early childhood. As all our MPS families will relate to our lives were changed forever and the road that lay ahead was going to have a lot of trials. I have always felt lucky to be given the chance of a bone marrow transplant with Jordan, as this is not an option with the other MPS diseases. When we discovered that Jordan's older sibling was a match we felt our path had been chosen and we had to give it a go. Unfortunately the first transplant didn't work and we had to repeat it. We soon settled into years of hospital appointments and operations; this was our life throughout the years when Jordan health was stable I have tried to do various fund raising as the MPS Society is such a big part of our lives and we have always wanted to help and support families who are in the same position as ourselves. I have run the women's 10k a few times and I have had various fundraising nights all for the Society.

As December 2014 came to an end I had decided that I needed to get fit so I registered for the women's 10k in Glasgow taking place on Sunday 17th May 2015. Originally this was a solo project but then I had an idea: why not rope in some of the other MPS mums. There are a few families who we have become friends with over the years so I set about recruiting some members. I managed to sign up Josephine (mother of Nadine) Kari (mother of Darryl) and

Emma (mother of Jess). The more money we could raise to find a cure for this cruel and devastating disease the better. Now I was on a roll on speaking to some family members and friends a lot of them were eager to join our now growing team; our other MPS mums managed to persuade a few members as well so TEAM GLASGOW was growing by the day. We were well on our way to a great fundraising opportunity.

I was overwhelmed by the amount of support we were receiving and even people who could not take part in the event were willing to take sponsor sheets. At first I thought maybe I had taken on too much but I was determined to get this working. 2015 did not start of well and in Scotland we had sadly lost 3 kids with an MPS disease. I can't sit back and watch this devastating disease claim more of our children. We need a cure and hopefully we can find one soon. We are moving in the right direction and a lot has changed in the 14 years since Jordan was diagnosed: who knows, maybe one day I will be able to say my son used to have Hurler Syndrome.

So on Sunday 17th May 2015, after a very wet start to the morning, the rain did stop as 53 women met up and were ready to hit the streets of Glasgow. Seeing us all together was quite emotional and I am extremely grateful to each and every one of you who were prepared to take the time and effort to raise money for a charity close to our hearts. A lot of the mothers I have befriended over the years also have

“We have always wanted to help and support families who are in the same position as ourselves”

children who struggle with their own diagnosis, so thank you for helping our MPS children.

So we ran and walked; some of them even had a snail pass them (you know who you are) but we finished. We weren't so lucky with the weather but that's typical Scottish weather for you. I had asked for the money to be in by end of June as myself Jim and Jordan were attending the conference in Coventry.

As the money came in I was starting to think this is going to be good. At the conference we handed over a cheque totalling over £10,000 but the grand total after the gift aid was added was an AMAZING £11,470.38.

I am extremely grateful to each and every one of you who pulled on your trainers that day. You are all very special people and I am privileged to have been given this chance to call you my family and friends

I would also like to thank a local business, owned by Wullie Haughey, for their donation.

Hopefully one day we will have a cure for all MPS diseases, so dream big and fundraise for the MPS Society

Jackie Mount

Thank you to all 53 members of Team Glasgow for their outstanding fundraising for the MPS Society!



Bake Sale Benefits



In the past 12 months almost four million people in Great Britain have baked for charity (8%), while nearly 15 million have bought food from a charity bake sale



Research published by the Charities Aid Foundation (CAF) found that bake sales in Great Britain helped raise an estimated £185 million for charities last year.



Most people (63%) agree that raising money for charity is a good reason to do something they enjoy while one in five people (20%) even agree that cake tastes better when it has been baked for a good cause!

Organising a Bake Sale to support the MPS Society

As you can see from the facts printed above, bake sales form an important part of a charity's fundraising. The beauty is in its versatility: it can be held at work, home or at an event, alongside other types of fundraising (such as a raffle), and can be as big or small as you wish.

However you would like to do your bake sale, we have a few tips which might help you on your way...

- * Contact the MPS Society for a fundraising pack, which contains posters, leaflets, balloons and more.
- * Offer a variety of sizes, from full cakes and pies, to individual cupcakes and biscuits
- * Cater for different dietary requirements, e.g. sugar-free, vegan, gluten-free, low fat
- * Decorate your tables and bring items packaged in pretty boxes and wrappers - a bit of bunting is always good too!
- * Make your pricing as clear as possible.
- * Get support from local bakeries and coffee shops - they may donate items to your sale.
- * Publicise your event on social media and put up plenty of posters in your local area to let people know when and where.
- * Consider time and place when planning your sale. Choose a time when people are likely to be out and about, such as a Saturday afternoon or alongside another big event.
- * Enjoy yourself!



Here's a couple of recipe ideas from MPS Society staff to get you inspired.

If you have a winning recipe you would like to share for MPS bake sales, why not post on our facebook page or email fundraising@mpssociety.org.uk.

Steve's Rich Orange Cake

This is an old recipe from Steve's grandma's cook book

- 6 oz. butter or margarine
- 6 oz. sugar
- 3 eggs
- 2 small oranges
- 8 oz. flour
- 1 tsp. baking powder
- ¼ tsp. salt
- A little milk

Cream together the fat and sugar and beat in the eggs one at a time. Add the grated orange rinds and beat well.

Sieve the flour, baking powder and salt, and fold into the creamed ingredients, together with a little milk to mix to a soft dropping consistency.

Put in a loaf tin and bake in a moderate oven for about 1 ¼ hours.

Charlotte's Melting Moments

A family fave and so easy to make!

- 200g/7 oz butter, softened
- 100g/3½ oz caster sugar
- 1 free-range egg, beaten
- 1 tsp vanilla extract
- 250g/9 oz self-raising flour
- 140g/5 oz oats

Preheat the oven to 180C/200F/Gas 4. Line a baking tray with greaseproof paper.

Cream the butter and sugar together in a bowl until light and fluffy. Beat in the egg until well combined. Stir in the vanilla and flour until well combined.

Divide the dough into 12 equal pieces and roll each piece into a ball. Sprinkle the oats onto a plate, roll the balls in the oats and put them on the baking tray. Squash the balls slightly with your hands.

Bake the biscuits for 12–15 minutes, or until pale golden-brown.

Email fundraising@mpssociety.org.uk

or

Call 0345 389 9901

to request a fundraising pack and for fundraising queries



Wear It Blue in Wales

To promote awareness of MPS, our local newsagent offered to collect donations for 'Wear it Blue Day'. As you will see from the photographs I have enclosed, they wore their t-shirts, decorated their shop with balloons and also had a raffle. My grandson Merlin Penny-Smith, who has MPSII, enjoys going into their shop to buy a pick-and-mix, so they know him well.

They managed to collect £110, which is amazing!!!!

Barbara Penny

Thank you to Andrew & Maria of Walker's News in Newport for supporting MPS Awareness Day.

Card Workshop

Dorothy Robinson, Nanny to Hannah Shannon (MPSIII), recently wrote in with a cheque for £220, which she raised by holding her 8th Card Workshop.

We were pleased to hear that the day went well, and as Dorothy said, "was filled with much laughter". MPS staff were also delighted to receive one of Dorothy's beautiful hand-made cards, which is currently on display in our office!

Dorothy has been a fantastic supporter of the MPS Society and we would like to thank her for all her hard work fundraising and raising awareness.

Grand East Anglia Run

My 4 year old grandson, Thomas, was last autumn diagnosed with Hunter's Syndrome. We knew for some time that he did have a problem of some kind. Then my first wife (Thomas's nanny) saw a picture on Facebook of a boy with Hunter's and it was obvious that Thomas was exactly the same. Since then his mum and dad have had an eight month whirlwind ride. The MPS Society have helped them survive the ride.

In December I decided to take part in the 10K Grand East Anglia Run on 3rd May. After training since January, I was determined to finish in under an hour. My confirmed start to finish time was 56 minutes 30 seconds, raising a total on JustGiving of £193. I am still running and next year intend to be a lot quicker.

Mick Yates

Thank you, Mick, and to Kayla Yates, who also ran the 10K. Together the pair raised a brilliant £530.71.

Good luck, Elliot!

On 10th October Elliot Moody will be running the length of the Lake District in one day to raise money and awareness for the MPS Society!

The race is 80km (50 miles) in length - which to give some perspective, is the distance of two marathons back to back! Starting at the very top of the Lakes in Caldbeck (above Penrith) and finishing in Cartmel (below Kendal) - the race covers the entire length of the Lake District.

Please show your support for Elliot's monumental fundraiser by visiting his JustGiving page: <https://www.justgiving.com/LakesInADay/>. Good luck, Elliot!



MPS Society Christmas Cards

With this edition of our magazine, you will have been provided with our Christmas Collection form, which includes our full range of festive greetings cards, as well as a money wallet, Magic Snow and Reindeer Food! All our cards and Christmas items are also available from our online shop - just visit www.mpsociety.org.uk to see our full range.

In addition to this, we are also one of the many charities that you can choose if you buy Christmas cards at Admiral Charity Cards (www.admiralcharitycards.org). This site offers a range of personalised corporate, business or office Christmas cards, where all the royalties will come to the charity of your choice! Postage is free to mainland UK and samples are also available.

Raise while you shop

Many of you will be starting to think about Christmas shopping (many of you won't, and will avoid any such thing until Christmas Eve!), and with people turning more and more to online shopping to cut out the stress of crowded shops, we wanted to remind you that just by simply shopping via sites such as Give As You Live and Easyfundraising, you can raise money for the MPS Society at no extra cost to yourself!

Shop with thousands of top retailers and find the perfect Christmas gift for family and friends, while helping to support those affected by MPS, Fabry and related diseases.

Take a look at their websites for more information:

www.giveasyoulive.com
www.easyfundraising.org.uk/

MPS National Draw 2016

The Society for Mucopolysaccharide Diseases is pleased to announce tickets are now on sale for our National Draw 2015, and if you have received this magazine through the post, we have included a book of tickets to get you started.

This really is a wonderful chance to help us make a difference to the lives of those affected by MPS and related diseases, and as well as being in with a chance of winning something from our fantastic list of prizes. The draw will take place at the Trustees' meeting on Saturday 28th November 2015.

We hope that you will support us by selling as many tickets as you can. Please invite friends, relatives and work colleagues to help sell and spread the word.

We have a huge list of really exciting prizes and there really is something for everyone!

★ **1st prize: 3 night family stay (inc. breakfast) at the Adina Hotel, Hamburg plus a 3 course dinner for 4 in the ALTO restaurant (transport not included)**

★ **2nd prize: £500 Millennium & Copthorne Vouchers**

★ **3rd prize: 3 night stay for 2 (inc. breakfast) at the Adina Hotel, Hamburg (transport not included)**

Plus: Red letter days vouchers, Cheltenham Racecourse tickets on New Year's Day, Ikea vouchers, Anfield stadium tour for four, cinema passes, personalised children's books, circus tickets, Fitbit Flex Wristband and many many more!

(Please visit www.mpsociety.org.uk for full prize list)

If you would like extra tickets to sell, please contact the office on 0345 389 9901 or email fundraising@mpsociety.org.uk, stating how many books you would like and your address.



Thank you to all our donors including...

Mark Hughes and friends raised €330 from their MPS stall, tombola and games at the Cambridge Arbury Carnival.

Scott Rogers raised €265 by abseiling down Berry Head in Brixham.

The Rotary Club of Westerham kindly donated €50 towards our 2015 Conference Volunteer funding appeal in support of a child from their area.

The Starlight Foundation donated a wonderful €576.33 to cover expenses for the children's entertainment and arts and crafts at this year's MPS Conference.

SM Brecker made a generous donation of €250 after staying with Holly Norwell at The Beryl Hotel in Wells.

The children at **Bursted Wood Primary School** held an afterschool homemade cake sale and raised a wonderful €296.53.

Loraine Patrick held a Stella and Dot Trunk Show and the commissions of €315 made were donated to MPS Society.

Riversdale House Surgery held a Wear It Blue Day and raised €74.

Jude Butler held a bonus ball competition at work, and also confessed for MPS with her friend Lynne and donated a total of €85.

Jo Addinall donated a wonderful €700 for Blake's Genes.

Portland Medical Practice held a Wear It Blue Day and raised €70.

Marie Renham took part in the KM Colour run to raise a wonderful **€543.19**.

Samantha McGinley raised €390 by taking part in the PPL Colour Me Rad Run.

Danielle and Tim Peach ran The London 10k and raised a wonderful

€1478.75.

Rachel, Niki, Hannah, Dan and Emma ran the London 10k and raised €987.50.

The Rotary Club of Sale kindly donated €150 towards a 2015 Conference Childcare Volunteer for a child from Sale. Rob Chilton met advocacy officer Debbie at Manchester Children's Hospital to hand over the cheque.

The Hilda Clarke Memorial Fund donated a fantastic €300 to pay for the cost of a childcare volunteer for our 2015 Conference.

Elizabeth Mee raised €40 from a coffee morning that she held as well as money raised from Sam's Music video.

The Rotary Club of Sturminster Newton kindly donated €100 towards the cost of a childcare volunteer at our 2015 MPS Conference for a child in their local area.

LV= kindly matched Jack Severy's and Darryl Brook's fundraising and donated a wonderful €1000 to The MPS Society.

Helen Coxon provided a donation of €93.73 for Blake's Genes which was raised from The Yorkshire Three Peaks Challenge.

The James Elliman Academy has kindly donated €521.37 from various fundraising events for The MPS Society.

Sarah Cutler, mum to Josh (MPSVI), raised an amazing €1,355.23 for MPS Awareness Day. Sarah did a brilliant job of getting local businesses involved, and our thanks go out to the Sandford Pharmacy, Sandford petrol station, St Martin's Primary School, Coll-Tec Services and the Purbeck School.

The Towersey Morris raised a wonderful €600 from dances held throughout the summer.

Jen Townson raised €318.75 from her Morecambe Bay Walk.

Chris White bravely raised €163.75 by taking part in a tandem skydive to raise money for The MPS Society.

Kelly Mitchell raised €240.71 from her completion of the 2014 BUPA Great South Run.

Hazel Swift donated €100 in support of Danielle Peach and her London 10k challenge.

Mrs Claire Simmons donated €100 in memory of Harry Brennan and June Gibbons.

Nick Trout, Ken Pierce and Colin Barton all completed the Prudential 100 mile bike ride in London and raised a total of €956.25 for The MPS Society.

Jibreel Arshad raised €1232.83 from taking part in Manchester's Sky's the limit bike ride.

Chris Long completed a 79 mile Yorkshire Tour cycle ride and raised €385 in support of Blake's Genes.

Val Turner donated €100 in memory of her son Richard, with the wish that it helps newly diagnosed families with advocacy support as they begin their MPS journey.

Mrs J Dine donated €10 from money collected in her collection box.

Spiffing Stationary has donated €150 raised from the sale of greetings cards and miscellaneous printing jobs.

Gordon & Mary Mellor donated €153.35 on behalf of all the generous people at the Grove Church in Horsforth in a loose change appeal.

Jeffrey Elliot raised €1743.26 by completing the Ironman Triathlon. The event entails 2.4 mile swim, followed by a 112 mile cycle and then a 26.2 mile marathon!

Donations

Mrs A Baker; Mr & Mrs Murray; Mr Chattha; Pamela Booty; Karen & Andrew Weedall; Mr & Mrs Brooks; Pharmaco Foundation Ltd; Elizabeth Beetham; Shone Owens; Sally Burrows; Susan Horner; Mrs L A Sutton; Mrs Joan Crespini; R E Perkins; Savvy Financial Planning; Judith Cotter; Mr & Mrs W Moffat; Mrs P Moffat; Val Turner; Dot & Lynne Ashcroft; Mrs S E Catterall; Mrs M Bennett; V Kaye; Ann Doyle; Mr & Mrs Bevan; S J Lewis; Mr & Mrs Bullock; Mrs Shirley Boore; K Watson; Carol Copsey; The Brock Family; Shirley Brown and friend; Karen Tylee; Susan Winters;

Alexander Kafizas raised £1132.50 from completing the London Triathlon which included a mile long swim in the River Thames!

'Marina & friends' raised a further £4955.65 from the sale of second hand goods in her shop in support of Sanfilippo disease. The cumulative total now raised by 'Marina & friends' is an incredible £146,784.13!

As a correction to the amount published in our Summer 2015 magazine, **Darryl Brook, Jack Severy and Mark Anderson** ran the Brentwood Half Marathon and managed to raise a combined total of £4454.52.

Pauline Bisson is a 64yr old Mother and Grandmother who recently took part in a tandem skydive to raise £770. Well done Pauline!

Jean Helen Davy; Martin Elliot; Rilwood Associates; Tayeeba Jalani; Gulum Sidat; Rashid Jakhura; David Sutton; Tony & Fran Eaton; Tony Muade; Donatas Mikuta; Geri Gray; Liz Merryweather; Kay Roberts; Montse Fernandez; Nic Crosby; Nigel Nicholls; Mrs E Mee; Mrs Hilary Lewis; Mrs E A Farwell; Mr Ian Knight; Wilma Robins; Molex Ireland Ltd; Mr & Mrs Hickman

In Memory

Mrs Deirdre Ann Coles; Jack Stuart; Dr Barry McGrattan; Mrs Margaret Munroe Greenhough; Martin Beecroft; Ross Challen; Richard Turner; Beryl Westhead; Mary Hood

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

Genzyme; Ellen Graham & Derwent & Solway Housing Association; J M Dine

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

R & K Dunn; S Littlelydyke, Norman Saville; M Newell, Marcia Tosland, S Bhachu; C Cullen; S Brown; I & A Hedgecock; V Lucas; S Winzar; D Forbes; P & R Shrimpton; G Simpson; D & S Peach; C & M Gibbs; Mr & Mrs Cock; A Dickerson; Manjit Kalsi; D J Holmes; P J Martin; P Summerton; A Weston; E White; CL Hume; A Byrne; Mrs D M Robinson; S Cadman; A Sullivan; J & F York; J Wilson; J & M Wood; A Tresidder; K Robinson; K Osborne; Molly Rigby; Mr Thompson; E Cox; M Peach; C Garthwaite; Raymond Arnold; J Ellis; I & V Pearson; William Cavanagh; A Sabin; Barbara Harriss; L Brodie; A Ephraim; J Dalligan; M Malcolm; E Mee; M Hahner; K Brown; E Moody; E Brock; M Fullalove; G Ferrier; E Parkinson; Margaret Leask; R Taylor; R Gregory; L Stillwell; R & K Henshell; K & S Bown; S & J Home; V Little; M Reeves; Michelle Boxell; S & D Greening; Z Gul; J Casey; J & V Hastings; J Winzar; Daniel Winzar; E M Lee; K Seeber; L Twaddle; M Morris; E Merryweather; M Boxell; J Garthwaite; A Thomas; N Miles; P Rennoldson; M Mould; P Berg; T Senior

Thank you also to all those who donate anonymously.

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.



THESE BOOTS ARE MADE FOR FUNDRAISING

Wicked Walkabout

Have fun, get fit and help support those affected by MPS, Fabry and related diseases by holding a Wicked Walkabout this year. All you need to do is get together with some friends, family or work colleagues, decide the route for your walk and start getting some sponsorship money in!

The great thing about this event is that you can make it what you want, whether you fancy a midnight walk around your local town or a forest hike the choice is yours. You can even decide how you would like to fundraise - you could ask each person for an entrance fee or a minimum sponsorship.

Just remember to request your fundraising pack and let us know what you are planning by emailing fundraising@mppsociety.org.uk or give us a call on 0345 389 9901. MPS T-shirts are available from our online shop and we have lots of free fundraising materials to help you on your way.

Help us to continue supporting the children and adults affected by these devastating diseases and funding vital research into treatments by walking for MPS!