MPS

Society for Mucopolysaccharide Diseases

Autumn 2010

What's Inside...

Update on Intrathecal Enzyme Replacement Therapy for MPS IIIA

Stories from the International Symposium on MPS Diseases, Adelaide

and a round up of events, news and treatment updates

Please donate to
www.mpssociety.co.uk,
phone 0845 389 9901
or post your donation
to our office, MPS House.

The MPS Society

Founded in 1982, the Society for Mucopolysaccharide Diseases (the MPS Society) is the only national charity specialising in MPS and Related Diseases in the UK, representing and supporting over 1200 affected children and adults, their families, carers and professionals. The MPS Society:

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

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

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

MPS & Related Diseases

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

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

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

Where does your money go?

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

Access to clinical management and palliative care

MPS Regional Specialist clinics

Support with disability benefits

Paving a child's way in accessing education

Upholding rights in employment

Advising on home adaptations

Bereavement support

Front cover photo:
UK MPS Group during the
International Symposium on MPS Diseases, Adelaide, Australia

Society for Mucopolysaccharide Diseases

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Magazine Deadlines

Winter	1 Dec 2010	Spring	1 Mar 2011
Summer	1 Jun 2011	Autumn	1 Sep 2011

Friend of MPS

Become a Friend of MPS to receive the Society's magazine and fundraising newsletter plus a range of other benefits. Contact us for more information.

IN THIS ISSUE...

Autumn 2010

MPS Children's Newsletter

In the Summer we were delighted to bring you the first ever MPS Children's Newsletter! A big thank you to everyone who contributed.

Our Autumn edition features a special section on 'Support to siblings'. Children who have a brother or sister affected by an MPS or related disease may recognise some of the issues and feelings addressed in this article and would like to write in and share experiences with us in the next MPS Children's Newsletter. The deadline is 1 December 2010.

For 2010 we launched an MPS Christmas Card competition, the results of which are published in the Children's Newsletter and in the MPS Magazine. Thank you to everyone who submitted their pictures. Many congratulations to the winner and runner's up too!

Please carry on sending us your stories and photos. We love to hear from you!

Email newsletter@mpssociety.co.uk Or via post: MPS Society, MPS House, Repton Place, White Lion Road, Amersham, HP7 9LP

Please get permission from your parents or guardian first!

CHIEF EXECUTIVE'S REPORT

Autumn 2010

Reflecting back over many years, the MPS Society has mourned the loss of so many loved ones and in the last decade has been here to celebrate the marketing of a small but significant number of treatments that are changing the course of MPS and related diseases and thereby improving the quality of life of many of our members. When I have written about these new developments over the years I have always been conscious of the Society's members still living in hope for their children with progressive neurological brain disease. When I hope many of you were focussing on enjoying what was left of the summer one young lady was making medical history for Sanfilippo Type A.

At the beginning of August 2010 Bethany Allen became the first person in the world to receive Intrathecal Enzyme Replacement Therapy for MPS IIIA in a "Phase I/II Safety, Tolerability, Ascending Dose and Dose Frequency Study of Recombinant Human Heparan N-Sulfatase (rhHNS) Intrathecal Administration Via an Intrathecal Drug Delivery Device in Patients With Sanfilippo Syndrome Type A (MPS IIIA)" underway at the Royal Manchester Children's Hospital. This trial will also be running in parallel with the Children's Hospital in Amsterdam. Over the coming months five more British children and six Dutch children with Sanfilippo A will receive Intrathecal ERT. Changing the analogy used by Neil Armstrong when he first set foot on the moon, "Bethany has taken one small step for Sanfilippo disease, and one giant leap for those affected by rare genetic progressive neurological diseases".

At the end of August the Society cemented this advance by hosting the first Expert Meeting on Sanfilippo disease and welcomed families, clinicians and scientists from 14 countries. This meeting was different from the National Conferences that the Society hosts every other year in that, through a series of short presentations, it exposed many of the research initiatives taking place around the world and enabled scientists and clinicians to learn from each other and to share their progress and aspirations with the families. Equally, three parents and two siblings spoke eloquently of their experiences caring for their children or brothers and sisters with MPS III. They could have left no doubt in the minds of the audience of the importance of the care these young people need today and their hope for the future.

In the coming months we will be planning the MPS National Conference for June 2011 at the Northampton Hilton hotel. There will be three parallel symposia addressing Fabry disease and all MPS and related diseases. This weekend is for all our members and their families so we want it to be right for you. If you have any thoughts or burning ideas on what you would like to hear during the symposia, please do let us know by the end of October so that we can be sure that the programme is as stimulating and innovative as possible. Whether you are a parent, sibling, clinician, nurse specialist, scientist or from academia, we would like to hear from you if you are interested in being considered as a speaker. Finally: SAVE THE DATE: 24 – 26 JUNE 2011 and come and celebrate the start of the Society's 30th Anniversary year!

Christine Lavery
Chief Executive

Become a

Friend of MPS

Would you like to show your support by becoming a Friend of MPS? We would welcome relatives, friends, overseas MPS families, professionals or indeed anyone interested in the work of the Society or the field of MPS and Related Diseases.

This would encourage us, help us plan for the future and bring about more public awareness for this group of rare, genetic, life-limiting diseases. You can also keep up to date with the latest information, news and stories.

Visit **www.mpssociety.co.uk** to download the application or phone us now on **0845 389 9901**.

Highlights from the Management Committee

The Society's Board of Trustees meet regularly. Here is a summary of the key issues that were discussed and agreed at the Management Committee Meetings held on **29 May** and **9 - 10 July 2010**.

GOVERNANCE
Between the two meetings Trustees reviewed and approved 29 policies. It was agreed that the Fundraising Complaints Procedure would be reviewed at the Jan/Feb 2011 meeting to further discuss the benefits of continuing the Society's subscription to the Fundraising Standards Board. It was agreed that the new Alcohol and Drugs Policy would be considered at the next meeting with the addition of an appendix. It was agreed that the review of the Child Protection Policy and Procedures would be deferred as this whole policy area is being updated into an overall safeguarding children and vulnerable adults policy. It was agreed that the review of the Data Protection Policy be deferred in order to accommodate major forthcoming changes to data protection law.

PERSONNEL
Trustees were advised that two advocacy officers had been recruited and would start work with the Society in early August and that the Grants and Corporate Fundraising Officer had resigned. Following the retirement of Advocacy Officer Linda Warner at the beginning of July after 2½ years at the Society, the CEO advised Trustees that there had been a small farewell lunch at the office and that she had been presented with a climbing rose from the Trustees as a token of appreciation for all her excellent work. On learning that Sue Cotterell is expecting a baby in November the Trustees offered their congratulations to Sue and her husband Steve. The Trustees agreed that maternity cover should be sought for Sue's post as PA to CEO/Office Manager.

RISK MANAGEMENT
It was agreed that there could be a reputational risk to MPS in relation to Jeans for Genes, but this was being minimised by the Trustees in agreeing to seek professional advice. There were no further changes needed to the risk register at this time.

TREASURER'S REPORT
Trustees advised the CEO that they approved of the new format for the accounts, year ending October 2009. Trustees agreed to seek advice on how frequently a charity would be expected to put out to tender for auditors. Trustees felt that the Society's current auditors, McLintocks, offer sound advice and good value for money, and that the Society has built up trust with them and they understand our business. It was also agreed that the Trustees propose McLintocks as the Society's auditors at the AGM.

JEANS FOR GENES
At the May meeting the Chairman gave an overview of the offer from Chronic Granulomatous Disease Research Trust (CGDRT) in which, as the trademark holders, they stake their claim to take over the running of Jeans for Genes (J4G). In this proposal the current arrangement with the four partner charities, of which MPS is one, would discontinue immediately after Jeans for Genes Day on 1st October 2010 and the three partner charities would receive a decreasing percentage of the income from J4G in the next five years in return for the MPS CEO and the CEOs of the other partner charities resigning their position on the J4G charity Trustee board by 16th July 2010. There was considerable discussion which continued to the July meeting when the Trustees, conscious that the deadline set by CGDRT was nearing, agreed to seek further professional advice.

CLINICAL MANANGEMENT
The CEO explained that no coroner's letter has been received in respect of the inquest touching on the death of one of our members with ML II.

The CEO updated Trustees on the current supply issues of Fabrazyme and strategies that are being implemented to manage this. The Society has been advising the EMA on future guidance to clinicians. In respect of MPS IIIA the CEO confirmed she had written to all known members to advise them of the forthcoming Intrathecal MPS IIIA clinical trial.

The CEO updated Trustees on the LSD paediatric National Specialist Commissioned service at Addenbrookes Hospital, Cambridge, and advised the Trustees that the Society had been asked by NCG to consult with its members clinically managed at this centre as to their preference for their children's follow up if the LSD paediatric service at Addenbrookes Hospital is decommissioned.

FAMILY SUPPORT
The Chairman fed back on the success of the International Symposium on Mucopolysaccharide Diseases held in Adelaide, Australia in June. Discussion took place on the importance of making it easier for patients travelling to these conferences accessing Enzyme Replacement Therapy whilst away from the UK.

The Trustees acknowledged the excellent work of the Advocacy Team and their handling of a large and active caseload at a time when they were short of staff.

Trustees approved the All Ireland Advocacy post shared with the Primary Immunodeficiency Association and learnt that one candidate had been identified.

COMMUNICATIONS
The Communications Officer spoke to her report outlining future plans for the MPS website and the range of MPS publications.

MPS RESEARCH GRANTS
The CEO advised Trustees that an anonymous donor had made a significant grant to fund an MPS III clinical trial for Genistein at the University of Manchester.

WHAT'S ON!

REGIONAL EVENTS

London Family Event, Science Museum
16 October

CONFERENCE EVENTS

MPS National Weekend Conference
24 - 26 June 2011

AWARENESS EVENTS

MPS Awareness Day
Sunday 15 May 2011

For more information about how you can help us celebrate MPS Awareness Day, please visit **www.mpssociety.co.uk**

MPS REGIONAL CLINICS

Birmingham clinic
26 November

MPS I Bone Marrow Transplant clinic (under 6's)
15 October

MPS I Bone Marrow Transplant clinic (over 6's)
22 October

Northern Ireland clinic
10 December

Bristol clinic
December (tbc)

Cardiff clinic
(tbc)

Please let us know if you're not able to attend an event for any reason
The MPS Society is delighted to offer subsidised places at a number of events throughout the year. If you book a place for yourself, and/or your family, but later find that you are unable to attend event after all, please do let us know. Sometimes we are able to offer your place(s) to other members and it means that MPS staff organising and attending the event aren't left waiting for you to arrive. We completely understand that sometimes it is unavoidable and last minute emergencies do crop up, but we would be very grateful if you could let us know by phoning the MPS office, out of hours number, or the contact number given to you on the event information sheet.

ANNOUNCEMENTS

New Members

Mr and Mrs Mawdsley have recently been in contact with the Society. Tillie-Mae has a diagnosis of Sanfilippo disease. Tillie-Mae is two years old. The family live in Hertfordshire.

Ms Susan Wilkinson has recently been in contact with the Society. Susan has a diagnosis of Fabry disease and lives in the North East.

Ms Sally Amos has recently been in contact with the Society. Her daughter Ella has recently been diagnosed with Sanfilippo Disease Type B. Ella is two and a half years old. The family live in the South West.

Emily was diagnosed with MPS I Hurler Disease in May. She is two years old and is receiving ERT at Birmingham Children's Hospital. The family live in Staffordshire.

Coleen has recently been in contact with the Society. Coleen has a diagnosis of Fabry disease and lives in the East Midlands with her family.

Mr and Mrs Mellalieu have recently been in contact with the Society. Gracie has a diagnosis of MPS IV Morquio Disease. Gracie is three years old. The family live in Wales.

Ms Little has recently been in contact with the Society. Jake has a diagnosis of Hurler Disease. Jake is one year old. The family live in the Midlands.

Mr and Mrs Van de Peer have recently been in contact with the Society. Caleb has a diagnosis of Hunter Disease. Caleb is three years old. The family live in the South East.

Deaths

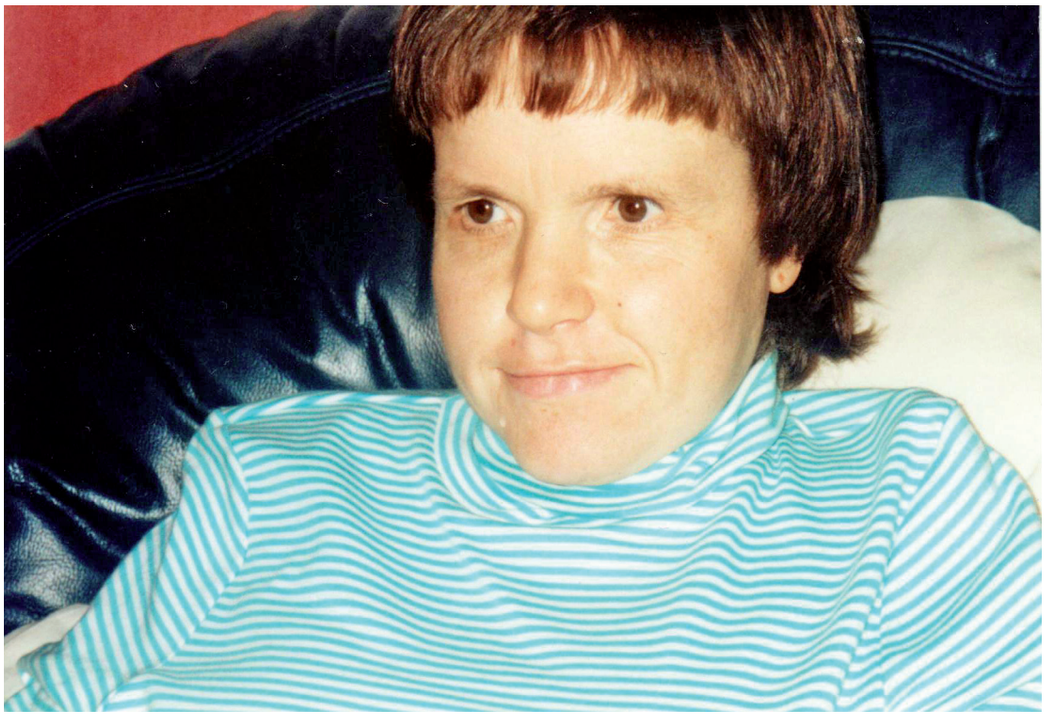
We wish to extend our deepest sympathies to the family and friends of:

Jade Hall who suffered from Hurler Disease and who passed away on 13 July 2010 aged 11 years.

Matthew Wright who suffered from Hunter Disease and who passed away on 4 August 2010 aged 22 years.

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

Congratulations to Lisa Nurse who recently celebrated her 39th birthday! Lisa has MPS III, Sanfilippo Disease



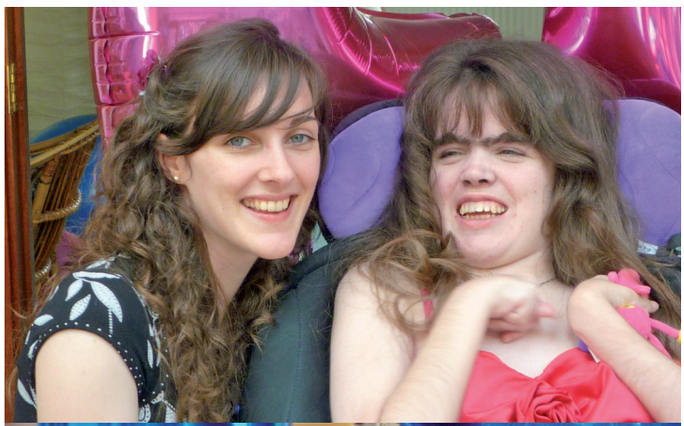
Amy and Daniel celebrate their special birthdays!

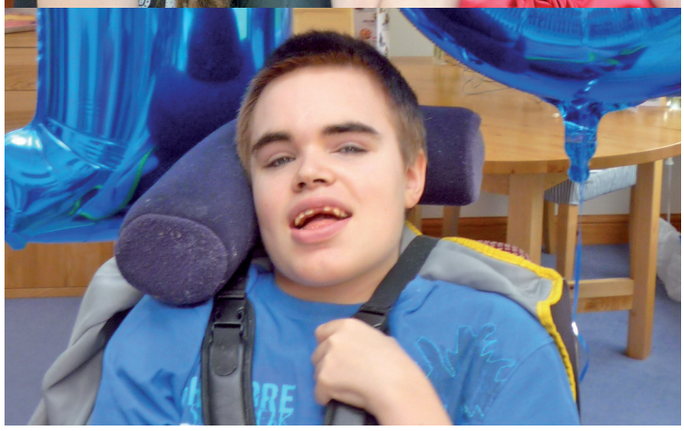
26th June 2010 was an exciting day in our home - it was a day that we thought we would never see following the diagnosis of two of our four children with Sanfilippo (MPS IIIB) around 15 years ago. We had a big all day party to celebrate Amy's 21st and Daniel's 18th birthdays.

We thank God for all the precious and happy years we have enjoyed and the day has been marked in our memories as a treasured landmark in the life of our family. Hannah and Josh were both home for the weekend to enjoy the day and help with all that needed doing.

We enjoyed a bustling morning with many friends and neighbours calling in to share in the fun. Amy looked beautiful, dressed in her favourite colour - a shiny pink party dress that Hannah had found for her. Both Amy and Daniel were happy and well on the day, enjoying the company and the buzz of activity around them. They loved everyone making a fuss of them and responded to being the centre of attention. Hannah had spent the previous week making 144 cup cakes which looked fantastic and tasted delicious too.

The afternoon was quieter with close family joining us. We spent the time in the garden and enjoyed a delicious BBQ. After the busyness of the morning it was nice to relax and enjoy same calmer time with Amy and Daniel to celebrate their special day. **David and Jan Donegani**





Meet the new members of our MPS team!

Lindsey Wingate

Hello, my name is Lindsey Wingate and I joined the MPS Society in August 2010 as an Advocacy Support Officer working with MPS III and MLII. I will also be working on research with MPS specialists and I hope with many of you, on the transitions teenagers face when they leave child centred hospital visits and begin adult centred hospital visits.

I have a background in Nursing, alternative therapies, counselling, social work and being Mum to 5 children and Nan-Nan to 2 grandchildren.

I like walking and running with my dogs and children, reading, meeting up with good friends, gardening and growing veggies and herbs, and listening to reggae and atlantic blues soul music.

I am really happy and fulfilled to be working here and having the chance to meet with you all and work with you and learn from you. Learning individual and collective life stories from members I have been fortunate to meet is an inspiration and motivates me to always give my best.
Lindsey Wingate l.wingate@mpssociety.co.uk

Rebecca Brandon

My name is Rebecca Brandon and I joined the Society at the end of July 2010 as an Advocacy Support Officer.

My background is in Local Authority Housing (I hear you all groan….) dealing with vulnerable adults and families and working with organisations to provide suitable housing for those client groups. Then I had a few years working with a private housing company and working with the Local Authorities. I now realise how frustrating it can be….

I have recently moved here from Dorset and have been busy settling into a new job, home and social life. I have just joined the local amateur dramatics group and do a spot of tap and ceroc dancing, yoga and pilates. Then I have to go to work!

I have had the pleasure of meeting some of you at the MPS III Conference in Northampton and found the whole experience very enlightening and was moved by the testimonies of the individuals and family members that are dealing with MPS and Related Diseases.

I hope that working for the Society I can in some way help to make life a little easier for the members and be a worthwhile and productive ambassador for the Society. **Rebecca Brandon** r.brandon@mpssociety.co.uk

Do you need help or advice from the MPS Advocacy Team?
Please phone us on 0845 389 9901 or
email **advocacy@mpssociety.co.uk**.

MPS Christmas Collection
available now!
www.mpssociety.co.uk

Christmas Card Competition

The MPS Society launched our 2010 Christmas Card Competition back in April. We invited everyone who was 15 years and under to send us a Christmassy drawing to be in with a chance of having their artwork appear on an MPS Christmas Card.

We would like to say a huge THANK YOU to everyone that sent us their pictures.

Everyone here at the MPS Society was amazed at the art work we received. It took us a very long time to decide whose drawing should win! A huge CONGRATULATIONS to Cora Halleron- Clarke who was our winning entry!

WELL DONE to our runners up who did a brilliant job. You should all be very proud. Here are the pictures from our runners up...

Our winner for 2010!

"Santa's Boots" by
Cora Halleron-Clarke, Age 11,
sister of Enola who has Morquio.

Runners up...

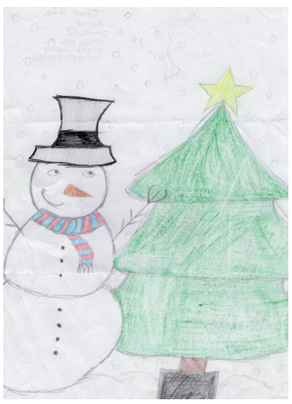
Archie Eaton
Age 9

Emma Shaw
Age 14

Bella Rudham
Age 4

Mia Fisher
Age 10

Will Summerton
Age 14



Lottie
Farrow

Isaac Eaton
Age 7

Tillie Kennedy
Age 14

Archie and Isaac's Infusion Diary

My children, Archie and Isaac, are on the clinical trial for enzyme replacement therapy for Morquio in Manchester. Over the last few months we have taken a series of photos documenting the experience and have put them together into a diary for the boys. The information is put together as a series of two photo diaries, one showing a typical infusion week and the other showing the tests that they have done every 12 weeks. They are written from the point of view of the boys telling the story of what they do in Manchester and will be used in school for them to help explain their experiences to their teachers and friends - and to help them understand the process better themselves. ***Anna Eaton***

Archie and Isaac travel to Manchester every week for treatment at the Children's Hospital. This is their story…

3.30pm - setting off
We have a condition called Morquio's syndrome which means that our bodies do not grow as well as most people's. The doctors have made a medicine that might help us to grow more normally and they want to try it out to see if it works.

Doctors can only try out new medicines in a very careful way, which is called a Clinical Trial. This is why we can only have the treatment in Manchester and not anywhere else and why we sometimes have lots of tests too.

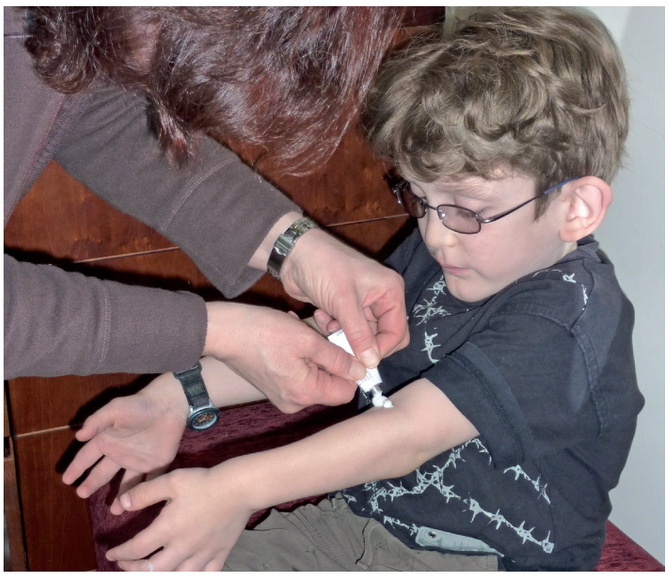
This is a diary showing what we get up to each week…

We usually leave straight after school.
7.30pm – arrive in Manchester

7.30am – breakfast
Because it is such a long way to go, we have to stay in a hotel the night before our treatment.

So, after stopping for tea on the motorway, we check into the Manchester MacDonald Hotel and go to bed. Sometimes we even have to share a double bed!

8.30am – getting ready



Before we leave the hotel Mum puts special cream on our arms so that it won't hurt when we have a needle put in. It takes time for the cream to work so this way we will be ready when we get to the hospital.

All ready to go!

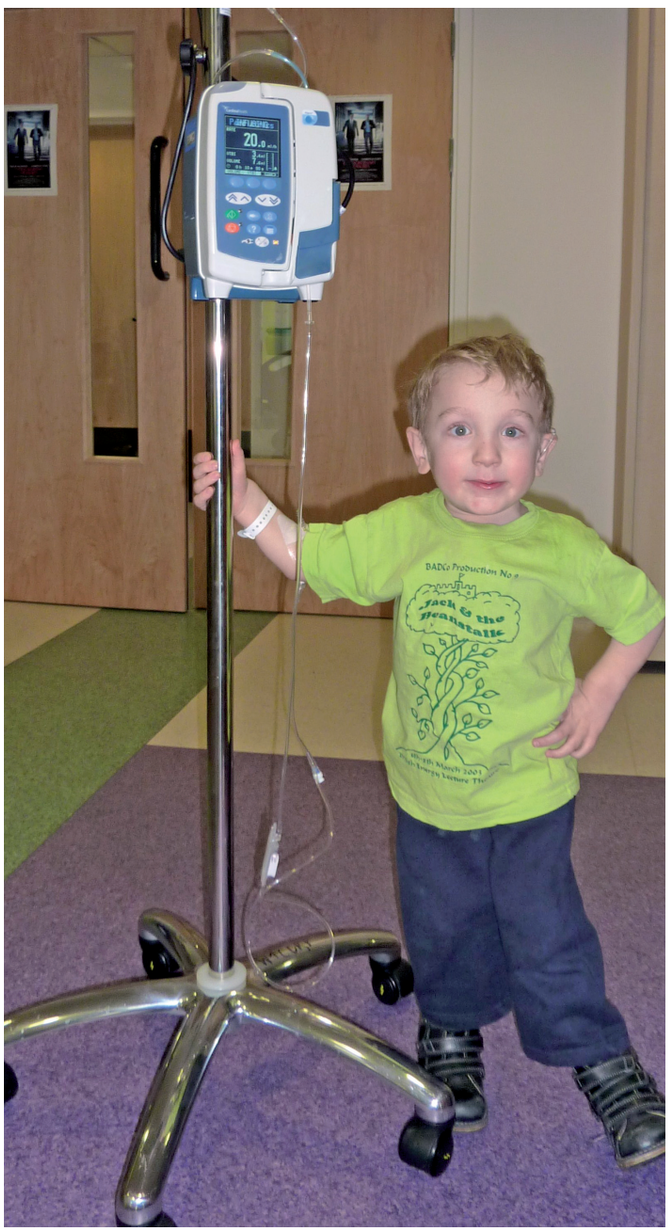
9am – at the hospital
We get to the Royal Manchester's Children's Hospital and go up to the Children's Clinical Research Facility on the second floor to meet our nurse, Kelly, and get started.

9.30am – Setting up
First the cream gets taken off and then a nurse puts a special needle called a canula into one of the veins in our arms. It's like having an injection except that the canula stays in your arm all day so that the medicine can be dripped in a bit at a time.

It doesn't hurt when the canula goes in as the cream is a local anaesthetic which makes that bit of your arm go numb. It's a bit uncomfortable when the cream is taken off though, as it is covered with sticky plastic which pulls at your skin when it is peeled off. This took a bit of getting used to at first but is not too bad now.

10am – starting the infusion
Next a tube is attached to the canula so that the medicine can flow in. This is what having an infusion means.

The medicine is controlled by a pump so that it drips in very slowly to make sure that our bodies don't react badly to it.



The pump and a bag with the medicine in are held on a special stand – we call our stands 'Stan' and they are like a best friend as they have to go everywhere with us – even to the loo!

11am – during the infusion
During the infusion, the nurses check our temperature and blood pressure every half an hour to make sure that the medicine is not making us ill.

The infusion last four hours so we do some school work, chat to the nurses and play games. We can usually move around where we like in the room – as long as Stan comes too!

12 pm – lunchtime
Lunchtime is usually a snack box with sandwiches, fruit and biscuits. We can eat and drink when we like whilst we are having the infusion but quite often it seems to make us not quite as hungry as usual.

1pm – the infusion continues
After lunch we can chill out and read or watch DVDs. We even get a visit from the clown doctors sometimes too!

2pm – end of the infusion
When the infusion has finished then the canula has to be taken out. This was uncomfortable at first too as it is held securely in place by more of the sticky plastic, which has to be peeled off carefully from around the needle.

We can have a little plaster on top to stop it bleeding afterwards but by the next day you can't usually tell where it went in. We will try and use the other arm next week so the same arm doesn't get too sore.

3.30pm – time to go
We have to wait an hour after the end of the infusion before we can go home, just in case we get unwell. Then it's back to the car and time to set off on the long drive down the motorway to Longhope. We'll get home just in time for bed!

Infusion Diary - extra tests week

Archie and Isaac's infusion diary showed what happens in a normal infusion week. But every 12 weeks they have to do a bit more - these pictures show their extra tests.

Day 1, 10am - ready to start
These pictures are from Week 36 – the 36th time we went for our infusion. We also did the tests in Week 12 and Week 24 too.

Here we are in the waiting room, getting ready to start. The first test is the walking test so it's good to rest a bit first to make sure we have all our energy for the test. We don't mind waiting though – the playhouse is great fun!

Day 1, 10.30am – 6 minute walk test
Before we start walking the nurses check how much oxygen there is in our blood, using a monitor which clips to our finger.

Today, Archie goes first! For the test itself we have to walk up and down a hospital corridor for 6 minutes without stopping. It's a bit boring but we can make it a challenge to see if we get further than we did last time.

Day 1, 10.36am – Archie's walk test completed
Six minutes later, Archie can sit down and rest. The physiotherapist helping with the test checks his oxygen levels again. This data gives the doctors useful information about how well our bodies are working. The doctors hope that the new medicine will make it easier for us to walk further. At the moment, having Morquio's makes us slower than other children, and we get tired more quickly too.

Day 1, 10.40am – Isaac's 6 minute walk test
We also have our blood pressure checked before the walk test, which you can see Isaac doing here.

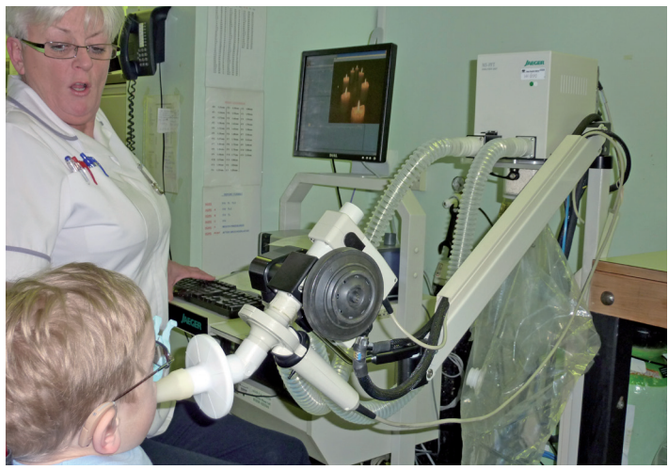
Day 1, 11am – weight and height
Morquio's makes us smaller than other children so we are weighed and measured to see if we have grown. As we are only growing a tiny bit at a time, they have to be very careful to measure it accurately. Here we are having our sitting height measured – from the bottom of our spine to the top of our head.

Day 1, 12pm – homework
In the middle of the day there is time to catch up on homework back at the hotel. We can have lunch and a bit of a rest before the next test too.

In the afternoon we have to go to a different hospital to do some breathing tests. We can't do it straight after the walking tests in case we are still out of breath from walking and it changes how well we can do the breathing test.

Because this is a clinical trial to test how well the medicine works there are lots of special rules like this, to make sure everything is as fair as possible.

Day 1, 5pm – breathing test



We blow into the machine which is connected to a computer. The candles you can see on the screen blow out if we blow hard enough into the tube.

There are lots of different pictures we can choose from which makes it more fun. The best one is a flying toaster which has wings and takes off if you blow really hard!

We usually sit in our wheelchairs to do this test as they are just the right size and shape for us to sit up properly with our feet supported.

The computer measures how hard we can blow so the doctors can tell if our lungs are getting stronger. Is there a bad smell? The peg on our nose is to stop any air escaping and make sure it all goes up the tube and gets measured.

We use the wheelchairs more often than normal on test days, so that we save our energy for the tests.

Day 2, 9am – 3 minute stair climb
The next morning it's time to start again! The rules say can't do the stair climb test on the same day as the walking test so we stay overnight in the hotel.

The stair climb is another way for the doctors to see if the medicine is making our bodies any fitter or stronger.

When we are at the top we sit and rest and have our oxygen levels checked again, just like after the walking test.

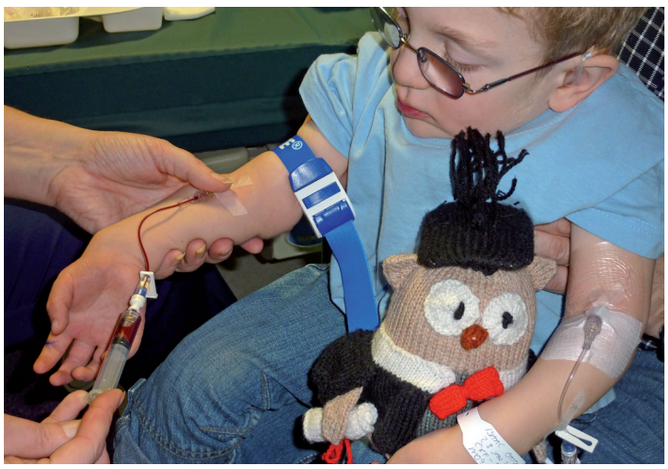
We have to climb as many stairs as possible in three minutes – the physiotherapists keep fit doing it with us too!

We usually make it right to the top – the 7th floor! If we get there in less than three minutes then we record the time it took and see if we were faster or slower than last time.

Archie holds the record but Isaac is catching up fast!

If we don't do the stairs or walk any faster than before then it doesn't matter, we just have to try our best.

Even if the results don't improve the medicine might still be working as without it we might start to find walking and stairs even harder than we do now.



Day 2, 10am – set up for the infusion
We have a canula put in one arm for the infusion like all the ordinary weeks. Only this time we have one put in the other arm too. This is so that the nurses can attach a syringe to it to take a blood sample whenever they need to.

You can see some blood being taken out of Isaac's right arm. Isaac always likes a cuddly toy with him whilst the canula's are being done.

Day 2, 11am – during the infusion
There are other children taking part in the clinical trial too. In week 36 we all got together for a group photo whilst we were having our infusion.

We still have our temperature and blood pressure checked regularly and do school work and play games just like during every other week.

But this time the nurses take lots of blood samples during the infusion as well, using the canula in our second arm. It doesn't hurt but it can interrupt our games a bit when they keep wanting to get at our arms!

The blood samples are important because the doctors can test them to tell if our bodies are working properly. As this is a brand new medicine they need to make sure that it doesn't do us any harm.

They also want to test our urine so we sometimes have to wee in a little pot when we get up in the morning in the hotel! The tests check that parts of our body like the liver and kidneys are not being affected by the medicine and that it isn't making us ill.

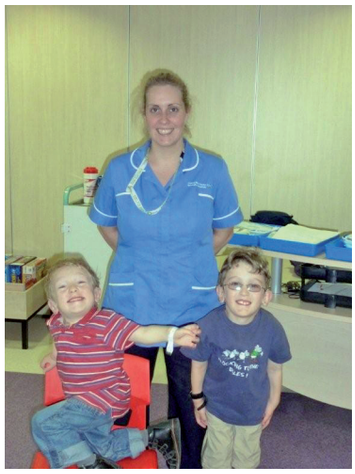
Day 2, 2.30pm – after the infusion
After the infusion we have to wait two hours this time so that they can keep on taking blood samples. It's sometimes a bit boring but we can play with our new friends and Mum brings fun craft activities for us to do.

Eventually it's all over and we can start the long drive home – it will be another late night.

Day 2, 4.30pm – time to go.

Thank you to Kelly!

This photo is of the boys with Kelly Burt, the nurse who has been looking after them every week in Manchester. Kelly has been the Clinical Nurse Practioner running the trial since the beginning and has just left to take up a new post in Birmingham. We thought it would be nice if you could include a few words of thanks to Kelly as she has been so supportive, right through from the pre-trial visits where we didn't know what was involved and indeed whether the boys would get on the trial. It has been especially nice for us to have that as we are travelling so far each week, as are a couple of the other families involved in Manchester. I know it was a hard decision for her to leave and although the handover arrangements have been well taken care of, we will all miss Kelly very much, especially the children. **Anna Eaton**



Diane's story

Before we knew about Fabry...

Even before we knew anything about Fabry, the children found it very difficult dealing with the 'normal' school life on a day to day basis. But we could never understand why. So we just plodded on the best we could. Tom is three years older than Laura, so we noticed this with him first, because Laura hadn't started full-time school at the time. Tom would have very regular bouts of vomiting, almost on a weekly basis, for 24/48 hours, but the doctors didn't know why, and just kept an eye on his weight. Then as he got older the headaches started daily, sometimes, this could be at any time of the school day, often I would get a phone-call to go and pick him up, then he would just sleep and sleep when we got him home.

There was never an explanation of why this would happen, if he got too hot, or ate too much he would just vomit, didn't matter where he was. (I have a hilarious story about an incident with this, but I'll spare you that for now). Anyway, to start off with the school was very understanding, but as time went on and as he got older the problem just got worse. By this time we had the diagnosis of Fabry and slowly things started to fall into place, and when Laura started full-time school, we could see the pattern repeating itself in exactly the same way with her. The two of them would be exhausted before even getting into school, because we had a mile to walk every morning. We learnt that Tom and Laura had to be careful not to get too hot, they couldn't join in with PE the same as the other kids, due to lethargy, and overheating.

It got to the point that the pair of them would be spending more time off school, than in school. We tried to explain to their respective teachers and the headmaster what Fabry was and how it was affecting them, but they couldn't really appreciate the extent of the problem, resulting in one of Laura's teachers actually reporting us to social services because of the amount of time she was spending off school. As you can imagine I was not very happy, I asked the teacher what would she like me to put in future letters of Laura's absence, if she really wasn't trusting what I already wrote. In the end I ran out of things to write, I just used to say that Laura or Tom was unwell with their Fabry and sent a Fabry booklet in for reference. What they did with the information then, was up to them. I have to say though, some teachers were more understanding than others.

So we struggled along for a number of years like this and we were getting nowhere fast, the two of them spent most weekends being ill in bed trying to catch up on rest, the same on the days that they were in school, when they got back after another mile walk. This was no life for small children, so we then took the major decision to educate both Tom and Laura from home. What a difference this has made. Tom has gone from year 6 all through high school and is now looking for part-time work. Laura came out of school when she was half way through year 3 and is now in year 9.

Their health improved drastically, they could rest when they needed to. Our pace of life has slowed down to accommodate what they can achieve in a day. If they have/had a bad day we wouldn't work, we knew they could always do schoolwork another day. They can now have a normal social life, instead of coming home and spending the evening in bed or the whole weekend in bed. I don't feel that Tom or Laura have missed out on anything with being taught at home, if anything it has given them a better quality of life than they would get had they stayed in mainstream schooling because they are in total control of what they can and can't do. If anyone was to ask them, they would certainly agree. **Diane Hughes, sister of Ian, featured right.**

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

Living with Fabry

Ian continues his series of articles written for the MPS magazine sharing his experiences of living with Fabry

Firstly I wish to thank those that took the time to contact me, I hope things are improving for you and I wish you a speedy recovery. I feel it is important to try and accept what is happening but we are all at different stages of the journey, and it is ok to be where you are as long as you are moving forward. When we lose our momentum, we stagnate, then we rust and can fall apart and in that instance we need to be restored.

Twenty years ago I did my first international truck driving job. We went out with one drop. It was empty beer cans from a company in Wrexham. As you can imagine I was very excited. The first and only delivery was in Hanover, Germany. The collection after this was from the Ford motor plant in Berlin (how lucky was I?). Well, not quite. At that time you went along what was called the corridor between east and west Germany. It was only 9 months after the wall came down so there were many trucks and cars from the east. One of those vehicles was the Trabant. You may have seen them on TV, a 2 stroke engine with the power of a hair dryer. Not being the most reliable of cars you either followed behind breathing a cloud of blue smoke or you saw them abandoned at the side of the autobahn (the corridor was at the time just two lanes with no overtaking for vehicles above 7.5 tonnes).

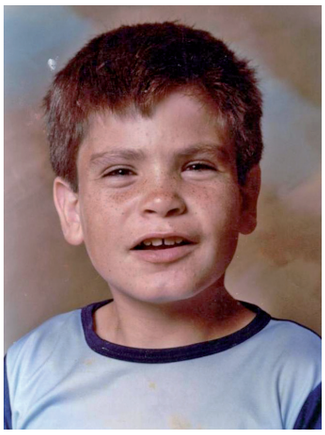
I was thinking about this journey recently and I likened having and living with Fabry disease to like being a Trabbant...

Yes there are some very fast cars out in Germany and you may well have one but in skilled hands you can get to the same destination in a Trabbant. I mean there may well be problems, you may need more time in a workshop but with tender care you can still run the race of life.

It also reminds me of the story of the tortoise and the hare (especially with Bugs Bunny's version). The hare got there in the end and in fact he won the race because he had dogged determination. We too are just like the Trabbant and the Hare. We might not be big, shiny and expensive with huge capacity, but we can do most of what other people can do just a little slower. And, yes, it takes a little longer.

It is good to set goals that are smart, specific, measurable, achievable and realistic. So ask yourself are the things we wish to do realistic and can we really achieve them?

Well, at last I have got my new car on Motability. It seems like ages since I ordered it. Until I was told about it, I didn't know that I could apply for a grant from Motability to help me get a car that would enable me to take my electric scooter with me and that I could go out alone and be independent. Well you can and I did get a grant and I now have a Mercedes travel liner. I only got five seats (the normal is 8) so now I have a great car that I can fit my buggy in and clear off on my own if I wish. Now I have my independence back.



Living with Sanfilippo

On these pages we feature a presentation by **Evelyn Jarvie, mother of Andrew who has Sanfilippo Disease**. Evelyn recently shared her experience at the MPS III Expert Meeting in August.

I'm Evelyn Jarvie. I am the mother of Andrew, a forty year old man who suffers with Sanfilippo syndrome. I'm here today to talk about some of the challenges Andrew and I have faced on what has been an exceptionally long journey – a journey of joy, heartbreak and at times, isolation.

To begin with, I have to say Andrew's young years were cherished as happy times with a few major problems. He was a happy boy and very able – always bounding with energy. He always had an enormous sense of fun; if he laughed, everyone laughed. Nonsense was the word.

Andrew attended local nursery school, primary school then moved to a special unit within a secondary school. The education system never failed him, always re-assessing his needs as his abilities changed.

Nevertheless, there was one major challenge which characterised Andrew's younger and teenage years and that was, never knowing exactly what was wrong with my son, when it was obvious that something was seriously wrong.

I remember being so frustrated at having no diagnosis. I remember thinking that finally being given an answer to that question would give me some sense of relief and I would be able to prepare for the future. When news of his condition finally came through when Andrew was 16 years of age, the doctor's prognosis was worse then I ever imagined. My son at this point was still able to enjoy life, cycle and play football with his friends in the park.

Yet, I had just been told he would be unable to speak and will be confined to a wheelchair within just a few short years. Even worse, he was expected to live for only another 10 years and no, there was no cure.

His future was unavoidable. In retrospect, it's difficult to conclude that if learning about MPS much earlier would have made the news more bearable. In any event, our only saving grace was that Andrew up until this point, had enjoyed a childhood surrounded by a family who were in blissful ignorance as to what lay ahead.

Some members of my family simply could not cope, knowing the road ahead of us. My husband succumbed to alcoholism, which brought added problems, just as Andrew was approaching his adult years, along with the more inhibiting symptoms of Sanfilippo appearing.

As well as losing his speech and mobility, Andrew became epileptic and incontinent. All these things appeared to happen over a very short period of time. He seemed to go through a stage similar to those with dementia. It was so difficult both physically and psychologically.

Added to this, was the fact that I was forced to go it alone as a single parent when alcoholism became too serious an issue to live with. How would Andrew cope? How would I cope?

Andrew needed me more than ever now. I had to jump in at the deep end of becoming a full time carer and a single parent to an adult with profound disabilities.

Input from all the services were now needed and I was thankful for all the help I was able to get, but it was also from this point onwards that I embarked on what would become the biggest learning curve I have ever been on.

Lots of good advice was given to me, but I also learned the hard way that professionals can make mistakes. I came to realise eventually, that I now knew how to care for a person with MPS and having the confidence of knowing that, this meant in lots of situations, I taught professionals and not vice versa.

If there is one lesson that I have learnt, when caring for Andrew, is that never be afraid to challenge, because as a parent, you are the one in the driving seat!

As time progressed, one service I welcomed with open arms, was a weekend respite facility in a local authority group home. This worked really well – I was in the driving seat, I was in control and carers followed my guidance and advice.

Andrew remained part of his local community, spending time with his family Monday through to Friday and used respite care every weekend. Likewise, Andrew had the use of Day Centre facilities during the week and he enjoyed this when he was able.

Respite was my saviour. Caring for a young man with profound special needs proved exhausting, especially during the more difficult spells, which Andrew naturally experienced due to his deteriorating condition. Life continued like this until Andrew reached the age of 29 years. Up to this point in his life, I hadn't appreciated how fragile my situation really was.

After all, Andrew's welfare rested solely with me and I too was becoming older. I began to think, if I reached the stage where I couldn't cope, Andrew would become even more vulnerable. I learned this lesson the hard way that particular year, when my emotional wellbeing was shattered following the deaths of two very close friends. I felt extremely isolated and feared that I didn't have the physical and emotional strength to carry on alone.

The local authority offered to care for Andrew full time in his respite home and after much soul searching, I reluctantly accepted this offer and viewed this as the best option for both of us, particularly at this time in my life.

To make this decision was horrendous for me and I can only describe the experience by saying simply that something had died within me that day. I made this huge decision under the belief that I would still have control over Andrew's welfare, but sadly this was not to be.

Many parents might be unaware that when the local authority cares full time for an adult with profound disabilities, the authority assumes, by legal default, the role of Guardian. In reality, that meant on so many occasions, my experience and knowledge of Andrew's welfare and care needs were being cast aside by some carers in the local authority accommodation where he was staying. I was horrified to discover, when I tried to challenge this, legally, I had no standing whatsoever.

I had to watch carers work against Andrew's interests and this was very stressful for both him and myself.

Andrew had been used to love, care, routine and structure in his life, but now, was having to cope with too many faces, too many attitudes and not enough continuity of care. Andrew was not happy and neither was I.

This was a situation that had to change – so back to the drawing board I went, having lost all faith in the care system.

The answer came when a new Director of Social Work took the helm, and who was willing to sit down, listen and work with me to find solutions.

After much planning, this dream was realised, when in March 2004, Andrew received the keys to his own tenancy and with his own dedicated team of carers.

We were able to ensure that this team received the specialised training in a wide range of issues relating to Andrew's condition. This was ranging from epilepsy to catheter care and even dietary management.

Such training doesn't happen overnight. It took months for staff to feel fully confident in caring for Andrew but when they did, I was finally able to relax knowing my son was being cared for properly and in the way he had been used to when I cared for him. A fine example of listening to Mum. I was still in the driving seat!

Dealing with professionals is so much easier now and when Andrew uses medical services, I find doctors, nurses, neurologists and psychologists all work well with me and the care team.

It needs to be said at this point, that Andrew's journey through life, has been extremely tough and what's more, he hasn't been able to talk up for himself for so many years now.

Words probably can't describe what he has had to deal with. For any parent, having to cope watching the progression of the disease, it is vital that services exist to help families when having to deal with the challenges inherent with MPS, not to hinder them.

I have been lucky to live in Fife, because the local authority's ambitious view for Care in the Community services has been realised over the past 20 years.

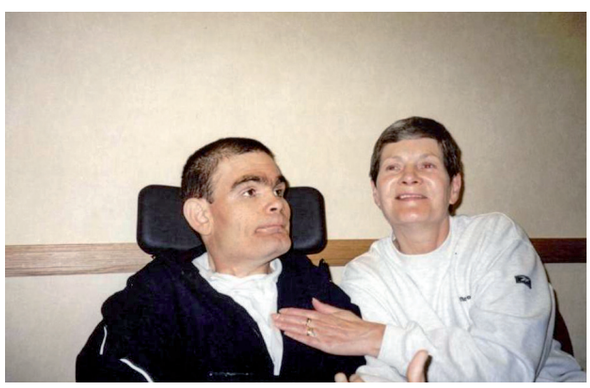
I would hate to think that families in the future would be denied the help that has been given to Andrew and I, given the current economic situation which all local authorities and ourselves will have to face in the future.

It is a worry that vital support services across the country could be hit hard by budget cuts. It's more important than ever for us as parents and professionals to speak up and retain this essential support. We have too much to lose if we don't.

There is so much more that I could tell you about the challenges I have encountered during Andrew's life, but I would be here all day.

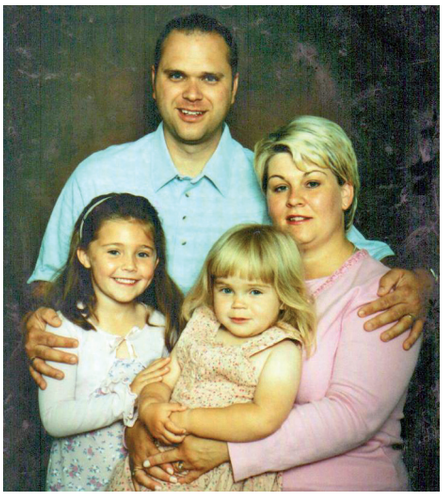
If I could distil my experiences down to one message of support for parents, it is this – always trust your own instincts, challenge decisions if necessary and above all remain in control, in the driving seat, and do not be afraid.

You are all very special parents and you are all carrying out a very special job with very special people!



Bethany is on the Intrathecal MPS IIIA Trial

This presentation was given by Bethany's father, John, at the MPS III Expert Meeting



Hello everyone, my name is John Allen and my daughter Bethany Allen has MPS III type A.

When I was asked to speak here today it reminded me of what we heard at one of the first conferences we attended after receiving Bethany's diagnosis. Professor Bryan Winchester had said then that tests had been done on mice with good results but they had to be injected directly to the brain. He said he believed within 10 years he expected to have something positive. I came away thinking what great news but it would surely be too late in coming for Bethany.

Here I am now ready to talk about an enzyme replacement study of which Bethany is the first in the world to try.

I had spoken to Louise Simmons from Birmingham Children's Hospital about the prospect of taking part in a study but had always wondered if it would be worth putting Bethany through all that.

A few days later Louise rang me at home saying she thought Bethany would be perfect for a study taking place in Manchester. We explained our reservations to her but we have always followed the advice of Dr Hendriksz and Dr Chakrapani's team at Birmingham Children's Hospital.

Louise sent us all the information and what was in store for Bethany and while reading it the phone rang. It was Kathryn Mc Bride from the Welcome trust at Manchester Children's Hospital. She asked if we had given any thought about Bethany taking part in the study. We had thought of nothing else.

She said they were looking to start the first person on the 15th July but understood if this was short notice. "We will be there" I said straight away, whatever it takes I will do. I have always said every day counts for children with Sanfilippo Disease.

When we arrived in Manchester we were greeted by the team of nurses Kathryn McBride, Fiona Heap, Ruth and Vicky and later Carol. We then met Dr Simon Jones who came in with a big BIG file and he explained the whole thing to me and his hopes for the study. His excitement struck me and it all seemed so positive. He did say that it would be a lot of strain upon the family and I said to him so is Sanfilippo Disease, we both agreed.

Next we met Stuart who did a questionnaire with me about Bethany. One question was 'if you ask Bethany to tidy her room would it be done in half an hour' and I said if I asked Jasmine who is 16 to do that I would be lucky! Other questions on certain tasks I gave my answers to and Bethany would prove me wrong later on in the day. You may think Bethany has lost different skills but she does have the habit of proving us wrong but in her own time and not when instructed.

Stuart is now adapting the tests so they are more suitable for children with Sanfilippo. The team is brilliant and I could not speak highly enough of them, they knew exactly what they were up against.

We returned to Manchester on 22nd July and Bethany had her operation on the 23rd, to fit a port device into the right side of her back to be able to administer the enzyme through. She has two scars on her back, and the port is fitted under the skin. A small price to pay for something that could change her life.

We were back in Manchester on 2nd August for Bethany to have a full body and brain scan for which she was given a general anaesthetic and while she was under, Simon had checked the port was working. It was all systems go! Bethany was fine, we were happy, the nurses were happy and Simon was like a kid in a candy store. The next day Bethany was going to receive the enzyme replacement. This was worrying me because Simon wanted her to be awake to do the procedure. How would we keep her still. I need not have worried as his sidekick Kathryn had been up all night and planned it like military manoveres. We all had our places and we all took our positions. Dr Ed Wraith had come to witness the moment, and it failed. The port had not worked. Bethany had to have the first dose of enzyme via lumber puncture. It was our first disappointment for the trial. After taking x-rays, which Kathryn and I can tell you is no easy feat, it was found that the tube had bent back under the port and had got a kink in it. How could this have happened, it had worked perfectly the night before.

I think I have caught the culprit. As the scar was healing I had noticed Bethany sticking her thumb under the area of the port and I guess it was her who sabotaged what would have been a successful mission!

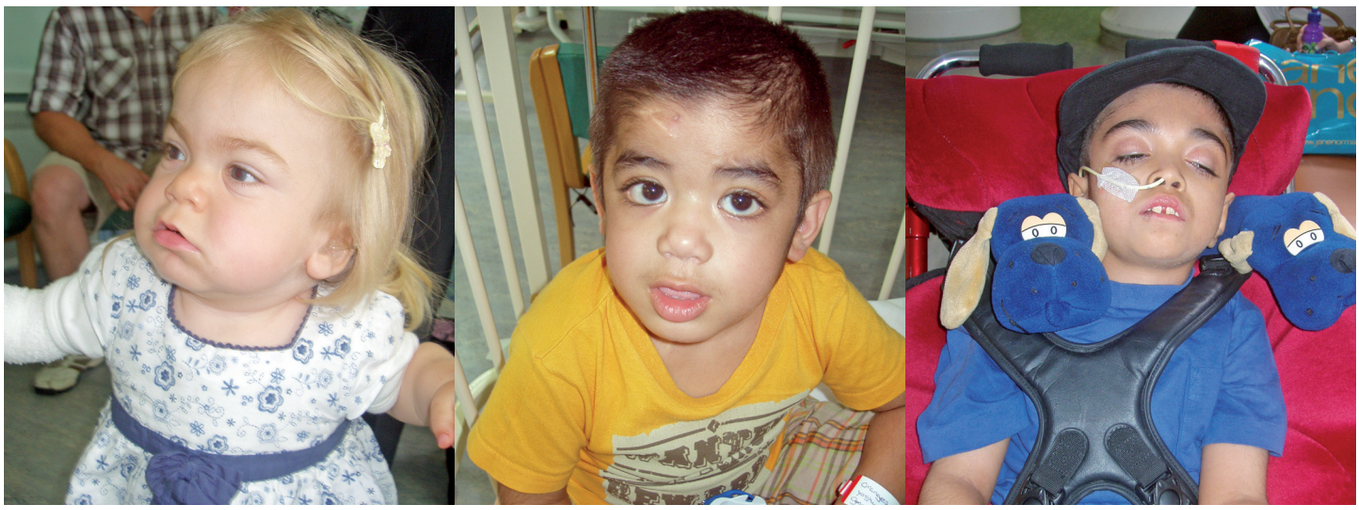
On 24th August all problems were sorted out and she is due to have a second lot of treatment on 31st August. That brings us up to the present day. As you will see Bethany is fine and in good health. I'd like to take this opportunity to thank Dr Simon Jones and his team for the hours of dedication and the MPS Society for sorting out accommodation and expenses and of course Shire, the pharmaceutical company, who have made it all possible.

Birmingham MPS Clinic

9th July

It was a beautiful sunny morning when I arrived in Birmingham for another busy clinic. I was very grateful to been given a separate consulting room, which gave families and individuals the opportunity, if they wished, to discuss any issues they had.

The clinic ran to time and went very smoothly, and it was lovely to meet up with familiar families and to meet families new to me and the MPS Society. I also had a tour around the hospital and was shown where our members receive their ERT, which gave me an opportunity to say 'Hello' to the children and parents who were not attending the clinic. I would like to thank all metabolic team at Birmingham Children's Hospital for their dedication, help and ongoing support.
Jolanta Turz *Advocacy Support Officer*



Photos left to right: Emily Bradshaw (MPS I), Muqadas Ilyas (MPS II), Pavan Tailor (Multiple Sulphatase Deficiency)

Camelot Family Weekend

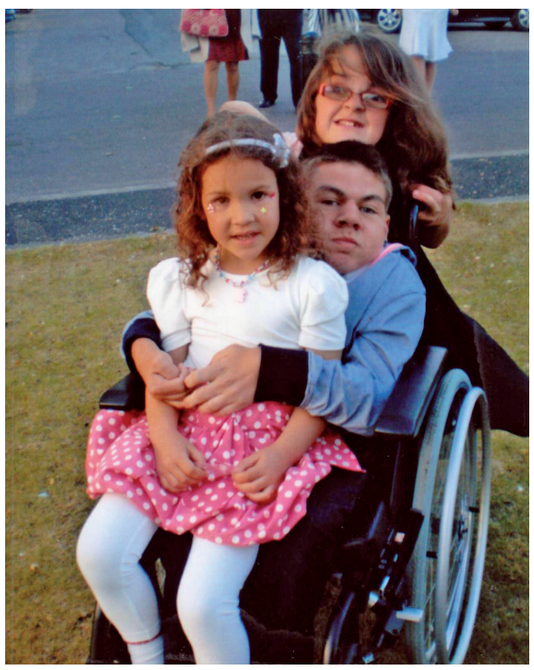
Thank you MPS for a fabulous weekend at Camelot!

"I would just like to say thank you once again to the MPS Society for a fabulous weekend at Camelot. It was wonderful to meet up with friends that we haven't seen for a number of years as well as having the pleasure of meeting new families in particular Gordon (AKA Duncan), Norma and Lewis. The weekend was superbly organised and the MPS Society provided excellent care for both children and adults.

Although we adore Alton Towers, it was a nice change to attend the AGM at a different theme park which also gave me the opportunity to meet up with a good friend from Liverpool.

Tara (MPS I) had a great time socialising with everyone although our older daughter Kate was unable to come with us due to her social engagements! However, Kate will be at the next conference in Northampton and is hoping to be a volunteer for the Society by then.

Once again, thanks to everyone involved with the weekend and look forward to the next MPS gathering." ***Maria Murphy***



International Symposium on MPS and Related Diseases

Adelaide, Australia, June 2010

Our journey began when a very excited group met at Terminal 3 Heathrow Airport to begin the long journey to Adelaide for the 2010 International Symposium. After a 14 hour flight we stopped in Singapore, which gave everyone the opportunity to stretch their legs and have a look around this amazing airport. After a stop over of 5 hours we continued our journey to Adelaide, this time the flight was much shorter but still some six hours before we touched down in Adelaide. For all of us we seemed to have 'lost' Sunday, as this was now early Monday morning. We made our way to the hotel which would be our home for the next seven days and despite being jet lagged, feeling rather grubby having been wearing the same clothes since Saturday, we tucked into a hearty breakfast!! Once we had eaten and changed our clothes we then set off to Port Adelaide for the Seahorse Farm.

As soon as we arrived at the farm we learnt that male seahorses are the ones which get pregnant and as you can imagine it caused lots of wishful thinking among the girls. We all had the opportunity to look at different species of these beautiful animals and even to stroke a real shark (it was gigantic)! On a way back we were very adventurous and caught the public bus to our hotel with all the other locals!

The following day after a good night's sleep we set off to Cleland Wild Life Park which is situated at the top of Mount Lofty just outside Adelaide. Everyone had a memorable day out which included holding Koalas, despite Jolanta telling us they were full of fleas!! We also fed the Kangaroos and walked close but not too close to the Emus.

On Wednesday we spent a beautiful winter morning walking along the river and finishing in the botanical gardens. It was hard to believe it was the winter as the sun was shinning and it was a very hot day. After lunch we registered for the conference, which we were all looking forward too. In the evening there was a welcome get together and we had the opportunity to hold a Koala again, sample Kangaroo burgers and even try fresh oysters.

INTERNATIONAL MPS SYMPOSIUM

The pace picked up the following day when we all set off to the conference. All our ambassadors attended the family conference and were busy writing in note pads information from each presentation and questions which they wanted to ask the appropriate speakers.

Over the next three days we had little time to spare as we darted from the conference hall to the main hall; we shared our understanding of each presentation and studied the posters on display. We were particularly interested in the MPS Society poster and also Great Ormond Street Hospital as this one included Jolanta and I in some of the photographs.

We listened to Jo Wilson-Smale as she described her life and future career expectations as she starts at Warwick University training to become a doctor, we listened to Megan Rennoldson introduce her life story as presented by Christine Lavery. I think we all felt very privileged in sharing in these young ladies' experiences and also being part of Team UK (as we became known!).

As the conference drew to an end with an amazing gala dinner, we all hit the dance floor, even Jibreel made it onto the floor, but would only dance the MACARENA, unfortunately the musician's didn't know this song, but it did not deter us from dancing it anyway!

The following day we set off on our long trip home, we travelled from Adelaide to Sydney, Sydney to Singapore and Singapore to Heathrow, the journey time totalled almost 30+ hours, but although tired we still kept our sense of humour! Particularly when greeted off the plane at Singapore Airport, where we were told we would be whisked off to a special lounge where we could eat, drink and relax. As we were taken up in a lift, past the workmen, along a corridor we passed the BA Executive lounge, then the Virgin Airlines lounge, then Singapore Airlines, we thought this is good, which one are we going too? Well that question was soon answered as we arrived in a very very small room with a few chairs, a vending machine and no food in sight!

We eventually boarded the final plane and set off for the 14 hour flight home, although Faye, Megan, Lois and Jibreel managed just naps, Jolanta slept all the way home, falling asleep after dinner and waking up just in time for breakfast!

Our thanks go to Keshini, Rosie and David our volunteers who worked so hard throughout the trip and particularly when we were tasked with getting from the domestic arrivals at Sydney airport to the international departure terminal. **Linda Warner and Jolanta Turz**

Photo right: UK Young Ambassadors - Keshini, Lois, Megan and Faye (front of picture)

My Visit to Adelaide, South Australia 19th – 28th June 2010 by Faye Longley

Faye (left of picture) with MPS friends

I remember coming home from work one day in January and finding the best message EVER on my answer machine! It was Sophie asking me to call her as there was the possibility of a trip to Australia for me! Obviously, I wasted no time in calling her back and getting my name down for that trip, I had always dreamed of going to Australia, now was my chance! I then had five long months to wait until Saturday 19th June finally came around!

Our flight left Heathrow Airport at 9.25pm and eventually landed at Singapore Airport 13 hours later (the first part of our journey). When we finally arrived at Singapore, a bit grumpy and tired due to serious lack of sleep, we discovered that mine and Jibreel's wheelchairs were not there to meet us when we got off the plane. Instead, we found two standard-sized wheelchairs on loan to us from Singapore airport to use for 5 ½ hours until our final flight to Adelaide! Anyone who knows someone with Morquio's will know that big wheelchairs with big gaps in the back are not the best idea in the world! We eventually got our own wheelchairs back after Christine had been arguing with the airport staff for about an hour and a half! They just didn't seem to understand what was wrong with the chairs they had given us!

After a quick Burger King and an even quicker freshen up and change of clothes, it was time to get ready to re-board the plane for the final leg of our journey. This time we had a shorter flight of 6 ½ hours which was a lot better than 13! However, all we wanted to do was sleep and the cabin crew seemed to do nothing but feed us, bringing round bananas every 5 minutes and waking us up with their torches... GRR! We finally arrived in Adelaide at around 7:30 on Monday morning.

This meant that we had a whole day to kill because, as much as we all wanted to, we knew if we went to sleep we would never get over the jet lag! So we ended up having a bit of breakfast at the hotel and then going to a local Sea Horse Farm which wasn't exactly what we thought it was going to be and ended up taking up about an hour of our time when we had the whole day to fill! We got the bus back to the hotel after going for a drink in a little cafe and ordering a few portions of chips to keep us going until dinner. That was when I first realised that Australian chips are the BEST chips EVER! It's funny how we had a 24 hour flight and it was virtually impossible to sleep, and 5 minutes on a bus and we were all dozing off! To while away the rest of the day, we went for a walk into Adelaide (the other end to where the Sea Horse Farm was) and had a mooch around the shops, some of which were pretty good! We had dinner at the hotel and by about 8pm, I was falling asleep, as were a lot of the others so I decided to go to bed. I think that night I was in bed and asleep by 9pm... That NEVER happens! Tuesday was our day at Cleland Wildlife Park which I think we all enjoyed. The highlight for me was probably cuddling Clover, the Koala Bear. Unfortunately, I was too small to hold her myself, even though she was one of the smallest bears they had! Keshini held her instead, and we had our photo taken together. Also that day we fed kangaroos, went through a couple of bird aviaries (where we found some birds with some seriously funky hair-dos!) and went through the reptile house.

That evening we went for a "fine dining" experience at Cos Restaurant which was about a 10 minute walk from the hotel. It was our first "main meal" in Australia and I think everyone will agree it was a good one! That was where me and Jibreel were introduced by Christine to Jocelyn, a 27 year old with Morquio's from the USA. Interestingly this was the first time she had met someone with the same condition as herself!

The following day we had a lovely walk along the river that ran behind the hotel (the weather was gorgeous) and ended up at the Botanical Gardens for lunch. In the afternoon we had to make ourselves look respectable for a "Morquio's photoshoot!" We then had some free time for a swim in the hotel's outdoor pool – divine! The evening followed with a welcome reception to the 11th Annual Symposium with a huge buffet including a chocolate fountain!

The next couple of days were spent in conference and we really enjoyed the evenings which consisted of a super Italian meal, family BBQ and gala dinner. The conference, although tiring, was an excellent experience and a chance to meet people from MPS Societies all over the world, particularly the Taiwanese, who seemed to take an interest in me, maybe it was my blonde hair and blue eyes! It was fascinating to meet so many different people with Morquio's and to have the opportunity to share our life experiences.

All too soon, it was time to re-pack our bags and head back to the UK. This time, we had two stops in the journey, one in Sydney (where we nearly missed our connection) and one in Singapore.

We arrived at Heathrow very tired but full of memories and experiences to share with our family and friends. Finally, I would like to thank both the MPS Society and Lady Gosling for giving me the opportunity to be a part of this trip; it is something I will remember for the rest of my life. **Faye Longley, MPS IVA**

Australia!

We met at the airport at 5.30 pm where we all checked in travelling together as one big group. This was slightly nerve racking as I didn't know anyone at all but there was one thing that united us all and that was MPS.

After meeting everyone and going through customs we had a long wait and this was the chance to get to know everyone slightly better as it was a long 13 hour flight to Singapore. Getting on to the flight was an issue as Faye's passport went missing! She kept saying to Christine that she gave it to Christine but Christine was certain she didn't get it.

The air stewardess mistook some of our group for children and we got children's colouring packs. After arriving at Singapore we had a 5 hour wait until our next flight, however we had an issue with getting the wheelchairs. The airport didn't seem to understand that Faye and Jib need their own wheelchairs. But the chair they gave us had a gaping hole in the back, which was not ideal since they both could fall through so eventually we got their chairs back. They both got big yellow stickers saying 'I am being assisted' to which Jibreel added; badly!

When waiting to board the next plane to Australia the steward that took us to the gate asked if we were going back to England? Laughingly I said I hoped this plane was going to Australia which thankfully it was. After another 6 hour flight and more plane food we landed in Australia. We were then taken by taxis to this amazing hotel and sat in a very comfy bar before being seated for breakfast. Faye pointed out that there was a bar made just for her and Jibreel as it was a seated bar which was at their height level. We had a breakfast in the hotel, and got our room keys. Faye and I were sharing a room and were meant to be given a disabled room with shower and bath but this was not the case. We got our room changed to another disabled room which had these facilities. There was an amazing view out of the window onto the river however we didn't think the room was very disabled-friendly that day.

It was a very tiring day for everyone and we went down to Port Adelaide to the Seahorse Museum. At the Seahorse Museum we were able to pet the baby shark and learn all sorts of facts about seahorses. We had an adventurous journey back when we took the local bus which stopped right outside the hotel. We met some very interesting people on this journey. Back at the Hotel, we were given freedom for a while, some of us went out and looked at local shops and others went to lie down. We all met again for dinner in the hotel, which was lovely. That night I sat up with some of the others and I listened to how the MPS started. I found the issue extremely interesting as I was able to expand my knowledge as well as see how much things have progressed, and how much advancement has been made for families nowadays. I began to realise that everyone on this trip was bound together by one factor and that was MPS and that it touched their lives in some way.

The next day, our group went up into the Adelaide Hills to Cleveland Zoo. It was full of beautiful views of South Australia and the hills. We also got to see Australian animals such as the Tasmanian Devil dingoes, hug and pet koala's and feed kangaroos and were even lucky enough to see a baby Joey, as well as all sorts of other animals such as reptiles and birds.

On Wednesday we had a lovely walk down the river where we were able to experience a different side of Adelaide. From here we walked all the way up to the zoo, which we considered going to but then decided against it, instead we went to the Botanical Gardens. We had a lovely lunch in the Botanical Gardens and then walked back through the city. We also signed up to the conference that day and Faye and Jibreel had a photo shoot. After that we had free time and we went swimming. Then we had a task on Wednesday night, we had to speak to 3 people we had never met before at the evening of the opening of the conference and one of them had to be an expert.

Thursday was the start of the conference, which was a very long but interesting day. The afternoon session was split, and I went to a different conference from everyone else but still really enjoyed it as it was more relevant to me. I was then able to meet other families from all over the world and one Australian family from Victoria. They have two sons with Sanfilippo who are still very able at the ages of 15 and 18. Their other son had never met someone who had Sanfillippo siblings before. I found this extremely sad to think how alone he could be feeling so I offered for him to get in contact with me.

Friday was another conference day, it was very interesting but it was also a very long and tiring day as there was just so much information to be taken in. Saturday was the same but after the closing session of the conference, we had free time so we all took off to the hot tub in the hotel where we relaxed before getting ready for the gala dinner. We all met in mine and Faye's room for hair and make-up before heading down to the gala dinner, even the boys! Not that they accepted our efforts to put makeup on them. Everyone looked so amazing and it was a lovely evening.

The way back we certainly had some adventures from losing the poster to nearly missing our connection flight in Sydney, to being taken to a special suit in the Singapore airport, to a lost bag at Heathrow. Like they say, nothing is ever easy but it was an amazing trip.

This was a very emotional but exciting trip and it was really rewarding watching how some people grew so much in such a short space of time. I had an amazing experience of making new friends. It was amazing to see us progress from a group of people that didn't know each other well to friends, as well as everyone learning something and developing as individuals. Thank you everyone for making it such a memorable trip.
Keshini Nonis, sister of Roshani (MPS III)

Megan's Adelaide Diary

Saturday 19th June 2010
We arrived early at Heathrow Airport and my parents had come with me. Faye had already arrived and later on we were to meet the rest of the Young Ambassadors. I handed my boarding pass and passport to British Airways, my parents said their goodbyes and left and I was off to Singapore Changi.

Sunday 20th June 2010
We arrived in Singapore at 10.30pm and were told that we had a five and a half hour stop so most of the Young Ambassadors wanted to shop and have a wander around.

After re-boarding the plane and another flight we arrived in Adelaide and arranged for two taxis from the airport to the Intercontinental Hotel. When we arrived at the hotel we had to sort the rooms and who would be sharing with whom for a week.

Monday 21st June 2010
Lois and I woke up at 8.00 because we had to meet the others downstairs at 09.00, I had a continental breakfast with Lois. Then at 12.00 we went to Seahorse farm and had lunch in a café near there. At 5.30 we had dinner in the hotel's Restaurant.

Tuesday 22nd June 2010
We woke up again at 08.00 to meet all the others downstairs and had breakfast at 09.00 with the Young Ambassadors including two volunteers whose names were Rosie and David. At ten we had to leave because we were going to a wildlife park to see different animals like emus, Tasmanian Devils and Dingoes. I held a koala bear and fed some kangaroos. After that we had a bite to eat before we went back and had a look round the souvenir shop for presents to take back home. At 7.00 we went to a restaurant which was only a walk away from the hotel and it was called COSE Restaurant to meet other people who came from other countries.

Wednesday 23rd June 2010
Woke up at 08.30 to be ready to meet everyone downstairs at 09.15. When we all finished breakfast we got ready to leave at 10.30 and to meet everyone at the front of the hotel. We went to the zoo and then had lunch in the Botanic garden. Then at 04.30 we all had to register for the conference, then after having a formal welcome and reception we had drinks at Riverside.

Thursday 24th June 2010
Got up early at about 06.00 for breakfast and to meet the others and then left for the first conference. After coffee we heard Joanne speak about MPS Type I. In the afternoon Joanne Evans and Heather Anderson talked about how they deal with their own illness.

Friday 25 June 2010
Woke up at 8.00 with Lois and had breakfast. After this I gave a brief introduction before Christine spoke on my behalf about Living with alpha Mannosidosis. I would to thank the organisers and in particular, Jenny Noble of LSD New Zealand and Wendy Boon from the Australian MPS Society for giving us the opportunity to tell my story.

Saturday 26th June 2010
Got up for breakfast at 7.00am. At 9.00 we all went to listen to talks on the value of patient feedback and what is happening about our hospitals in England.

Then it was the last night of the conference and everyone who attended came to the Gala Dinner. There were three Awards presented and Ed Wraith won one of them which was good. We also had a dance which was really great and I enjoyed myself too.

Sunday 27th June 2010
We had to be up and ready by 7.00 then have breakfast before going back home to England. We left at 08.00 so we would be ready to catch the plane at the airport at 08.45.
Megan Rennoldson (Mannosidosis)

Young Ambassadors and MPS Staff in Adelaide

An Overview by Lois Pack

11th International Symposium on Mucopolysaccharide and Related Diseases Adelaide 2010

In general I found the conference to be very interesting and enjoyable. I enjoyed the chance to get to meet and know other people who have experience with other MPS conditions. Attending the conference gave me the chance to learn more about MPS I HS that I was born with. I also enjoyed the outings as they helped me to learn about the inhabitants of Australia.

My favourite excursion was the visit to Cleland Wildlife Park, where we got the chance to get up close to Australia's indigenous wildlife, with my favourite part being the chance to be photographed hugging a koala. My second favourite excursion was our walk alongside the Torrens River to the Botanical Gardens due to the lovely scenery of the walk which gave me the opportunity to see Adelaide.

During the Conference Welcome Reception I met several people (such as John Hopwood) who had experience of treating people with MPS and related diseases. Of all the talks and presentations at the conference I managed to find many things to be of interest and relevant to my experiences with MPS I HS.

On the Thursday the talks regarding the diagnosis and treatment of MPS diseases were of interest. Joanne's talk on her experience of ERT made me glad that I was diagnosed when I was three; with her talk in particular reminding me that all potential "cures" have side-effects. The talks on Thursday afternoon relating to the skeleton interested me as I have had many skeletal problems, in particular with my hips and legs. I also found the presentation on managing carpal tunnel syndrome interesting as I have had both my carpal tunnels operated on at least once.

On the Friday I found the outcomes of treatment of MPS diseases with BMT and ERT to be of interest, in particular as to how the two treatments are combined and that early diagnosis is essential. The talks that I found the most interesting and relevant were those on anaesthetics and the management of heart conditions in people with MPS. As I had my aortic valve replaced five months before attending the conference, I particularly found the ultrasound pictures and the experience of the speaker relating to the degradation of the heart valves fascinating. I am also very proud that my room mate at the time, Megan Renoldson, had the courage to stand up and introduce the presentation relating to her experience with Alpha-Mannosidosis.

On the Saturday I found the talks and presentations relating to how people with MPS are transferred from child to adults services around the world to be of interest in learning of other's experiences in comparison to mine. I found the Gala dinner at the end of the conference to be an enjoyable conclusion to my time in Adelaide, Australia. All in all I had a very enjoyable and interesting time at the conference in Adelaide. By attending the conference, I feel as though I have benefited from attending the conference by coming away with a greater understanding and awareness of MPS diseases, along with the effects of potential treatments.

Photo left: Lois and her friend the koala; Photo right: The Gala Dinner

MPS III Sanfilippo Expert Meeting

27th - 28th August 2010

August was an auspicious month for the MPS Society as it saw our very first Expert Meeting on MPS III Sanfilippo Disease.

This inaugural event took place at the Hilton Northampton from 27th to 28th August 2010 and was the largest gathering, hosted by the MPS Society, of Sanfilippo sufferers, their families and professionals that has ever taken place.

This expert meeting offered individuals, parents, partners, carers and professionals alike the opportunity to hear state of the art talks on the clinical management of individuals affected by MPS III as well as updates on the latest research developments and potential therapies.

With thanks to Ann Marie Watson, Keshini Nonis, Evelyn Jarvie, Hannah Donegani and Karen Robinson, those attending the conference were also provided with a first hand account of what life is like on a day to day basis when a family member is affected by MPS III Sanfilippo Disease.

The conference programme was designed in such a way as to offer those attending the opportunity to meet and talk informally with professionals, and for individuals, parents, partners and carers to share their own experiences with others.

Alongside the main conference was a separate social programme designed for children and vulnerable adults affected by Sanfilippo Disease as well as their siblings, aged 17 and under. This event's programme allowed everyone to have fun whilst also providing the opportunity for them all to talk and make friends with others in similar circumstances.

There are, as always, plenty of 'thank you's' to be made. Like all our MPS events and conferences, nothing would be possible without the support of our amazing volunteers to whom we would like to send our sincerest gratitude.

On these pages are a selection of photos from the event.

We would also like to thank all of the professionals who gave up their valuable time to speak at this conference and for providing us with an insight into the exciting progress made within research and development.

All of us at the MPS Society hope that all who attended this MPS III Sanfilippo Expert Meeting were able to leave with fresh insights and a renewed hope for the future.

In the next edition of the MPS Magazine, there will be an update on research and treatment initiatives regarding MPS III. We will also provide further information on fundraising initiatives that may help us to fund further research into Sanfilippo disease. The Winter 2010 edition will also include feedback on the recent International MPS Network Meeting.

"Just a quick note to thank you and all the MPS staff and volunteers for an excellent two days. We all enjoyed it immensely although I think Will was rather worn out! Please pass on our thanks to everybody."
Tim & Sally Summerton (Sophie, MPS III)

"Thank you all so much for yet another fabulous conference. This was just fantastic, with so much new news about Sanfilippo. We felt heartened to hear all that was going on, and privileged to be part of what was a really important event. We enjoyed the dinner and the children had a great time on the sibling outings - especially Megan, as it was her first time taking part. Thanks again to you and all your colleagues."
Jess and Tim (Jamie, MPS III)

Please tell us how you found the conference.
We would love to hear from you! **newsletter@mpssociety.co.uk**

The Inherited Metabolic Disorder Unit at University Hospital Birmingham

ADULTS who have grown up coping with Inherited Metabolic Disorders (IMDs) can now access a new service at Selly Oak Hospital, part of the University Hospital Birmingham NHS Foundation Trust. This is the first step in developing a Rare Diseases Group at the Hospital, which will provide highly specialised care to patients and their families, facing the challenge of managing these rare conditions.

The team includes Dr Tarek Hiwot, (Consultant in Inherited Metabolic Disorders), Jane Lodwig, (Clinical Nurse Specialist), Kate Peers (Nurse), Louise Robertson (IMD Dietitian) and Rishender Singh, Pharmacist. Our IMD Service Co-ordinator Liz McAlister is due to start in September. We have also secured designated physiotherapy time for our LSD patients and are currently working on securing funding for other therapies/ resources. We also have a volunteer (Maggie) and a medical secretary (Jeanne) who make sure that everything runs as smoothly as possible in the office.

The IMD Unit at Birmingham Children's Hospital (BCH) is one of the leading units in the United Kingdom and looks after many patients with IMD.

Improving medicine means many patients are living longer and the need for a specialist Adult unit became apparent. It is not an ideal situation for adults to be attending their Outpatients appointments at a Children's Hospital or travelling long distances in order to access treatment.

The Adult IMD Service has been under development at UHB since 2007, in conjunction with the established Team at BCH. Of the patient population, >80-85% of patients have had a diagnosis made in childhood and are transferred to Adult Services at the age of 16, and a further 15-20% represent new Adult referrals. Currently, the IMD Service looks after > 200 patients with IMD and the catchment area is the entire West Midlands and surrounding region (population > 5 million). The UHB Team works very closely with colleagues at BCH and aims to provide seamless care for all patients with IMD.

We have a weekly joint clinical meeting for both Adults and Paediatric patients and the on-call service covers all aspects of clinical IMD.

There are many Inherited Metabolic Disorders. The most common is Phenylketonuria (PKU), with approximately 5-6 patients born with PKU in the West Midlands each year. Screening started in 1969, so the oldest well treated patient with PKU is coming up to 40 years old. A strict low phenylalanine diet is now recommended for life. Patients require ongoing support to help them manage their condition at home, as well as regular blood tests and dietary advice.

The IMD Service also looks after the full range of patients with IMD including Galactosaemia, Homocystinuria, Fatty and Oxidation defects, Mitochondrial disorders and Lysosomal Storage Disorders (LSDs) and is currently developing its enzyme replacement therapy (ERT) service, with the support of NCG (National Specialist Commissioning Group). In April 2010, Birmingham became the most recent NCG-designated service for Lysosomal storage disorders and has now commenced ERT at UHB, with the first patient already moved on to Homecare. The development of the service for patients with LSD's is a real opportunity to create something which really meets the needs of patients, and the Team at UHB welcome input from patients, their families and carers. We aim to develop a comprehensive service, which addresses all the needs of patients and helps them manage these complex conditions in a way which works for them. We welcome input and ideas from patients and encourage them to contact us with any questions or ideas about their treatment.

We are looking to develop close links with colleagues at local hospitals, providing expert advice and emergency regimes to help them manage emergency admissions where appropriate.

The Team can be contacted via the UHB Switchboard: **0121 627 1627**

Photo next page of the team, left to right:
Louise Robertson (IMD Dietitian), Kate Peers (IMD Nurse), Jane Lodwig (IMD Clinical Nurse Specialist), Dr Tarek Hiwot (IMD Consultant).

So, what has been achieved so far:
2008/09

- Appointment of a Consultant, Dietitian and Specialist Nurse
- PKU Patients transferred from BCH
- NCG approval for Adult LSD Business Case
- Weekly clinics for new patients and follow ups
- Monthly LSD clinic

2009/10

- Transitional Care Policy in development with BCH. General metabolic patients (aged 12-18 plus those already adult) commenced on Transition Pathway. Fortnightly Transition clinics at UHB and BCH.
- Monthly LSD clinic
- 6 monthly joint clinic with Dr Atul Mehta (Royal Free Hospital)
- Pharmacist appointed (Part time)
- LSD nurse appointed March 2010
- 1st ERT patient commenced treatment at UHB in Feb 2010
- IMD Service co-ordinator appointed July 2010

Where to next?
The Team has done a lot of groundwork preparing for the transfer of larger numbers of patients with a wide range of presenting metabolic conditions, and also for the development of Enzyme Replacement Therapy at UHB. We know that there are significant numbers of metabolic patients locally who do not currently have easy access to treatment. These patients may have to travel to other centres for their treatment or they may not be under the care of a Metabolic Team at all.

We aim to continue to develop our Service so that the people of the West Midlands can have access to a local, specialist Metabolic Team throughout their lives… supporting them in hospital and at home and enabling them to access to the very best in specialised care

Jane Lodwig
Clinical Nurse Specialist IMD

Nephropathy in Fabry disease: recent data from the Fabry Registry

Emma James DPhil – Senior Project Manager, Global Registry programme, Genzyme

It is estimated that up to 11% of adults have chronic kidney disease, and the majority of cases are caused by diabetes and/or high blood pressure [1]. There are, however, numerous other less common causes of renal dysfunction, including genetic disorders such as Fabry disease. This article will describe a study by Ortiz and colleagues who analysed data from the Fabry Registry, focusing on both men and women with chronic kidney disease [2].

Overview of chronic kidney disease

Chronic kidney disease is a progressive disorder staged from 1 to 5 depending upon the 'glomerular filtration rate', which is a measure of the rate at which the kidneys filter waste from the blood. In chronic kidney disease, the filtration rate slows and 'waste' accumulates leading to clinical symptoms. In many cases, the first sign of kidney disease is the appearance of albumin (a protein found in the blood) in the urine, known as albuminuria. As kidney impairment worsens, the amount of protein in the urine may increase, known as proteinuria (typically stages 1–3). It should be noted, however, that not everyone with chronic kidney disease has high levels of proteinuria (known as overt proteinuria), and the association between kidney function (filtration rate) and protein levels in the urine is not fully understood. By the most advanced stages (stages 4/5), kidney function is severely impaired, or the kidney may fail to function, and this is termed 'end stage renal disease'. Patients with end stage renal disease need dialysis and may need a kidney transplant.

Chronic kidney disease is common among people with Fabry disease, particularly males [3, 4]. An earlier study of men with Fabry disease who had not received enzyme replacement therapy (ERT) reported that 24 of the 105 men (25%) developed end stage renal disease at a median age of 47 years; none of the men survived beyond the age of 60 years [5]. More information is needed about the progression of chronic kidney disease in Fabry patients, so that it may be detected early and prevented from progressing to kidney failure.

Kidney disease in men and women in the Fabry Registry

To investigate the features of kidney disease in people with Fabry disease, Ortiz and colleagues recently analysed renal data from men and women in the Fabry Registry [2]. The analyses included people over the age of 18 years who had not received ERT. As of April 2007, a total of 1262 untreated adults had renal data available, including 585 men (46%) and 677 women (54%).

The majority of people had stage 1 or 2 kidney disease (Table 1). However, more men than women had kidney disease at stage 3 or worse. In people aged 40 years or above, 45% of males and 20% of females had kidney disease at stage 3 or worse.

Table 1. Distribution of patients with kidney disease in the Fabry Registry by disease stage [2].

Table 1.

Stage	1 Normal kidney function but with protein or blood in the urine	2 Normal kidney function but with relatively high amounts of protein in the urine	3 Moderately reduced kidney function	4/5 Very severe reduction in kidney function, or end stage renal disease
No. men (%)	275 (47%)	144 (25%)	88 (15%)	78 (13%)
Average age	30.2 years	42.1 years	45.6 years	43.8 years
Age range	18–68 years	19–66 years	20–72 years	21–79 years
No. women (%)	307 (45%)	281 (42%)	67 (10%)	22 (3%)
Average age	35.8 years	46.8 years	56.5 years	46.6 years
Age range	18–70 years	18–83 years	24–82 years	20–74 years

A total of 606 people had 24-hour measurements of their urine protein levels recorded in the Fabry Registry [2].
o A significantly higher proportion of men than women with chronic kidney disease had protein in their urine.
o Men and women with overt proteinuria were often at an advanced stage of kidney impairment; nonetheless, 11% of men and 28% of women who had stage 3–5 kidney disease did not have overt proteinuria.
o Although protein in the urine is an indicator of kidney impairment, symptoms do not generally emerge until proteinuria levels reach a certain level known as the 'nephrotic range'. A total of 22 men (7.3%) and 11 women (3.6%) had proteinuria levels in a nephrotic range.
o Ninety-three people (31 men and 62 women) with stages 1 and 2 kidney disease had albumin in their urine, although they did not have overt proteinuria. This supports the suggestion that albuminuria often precedes proteinuria in people with chronic kidney disease.

A total of 52% of men and 47% of women with kidney disease stage 1–2 had abnormally raised blood pressure. Furthermore, in people aged less than 40 years with stage 1–2 kidney disease, a significantly higher proportion of men (248; 51%) had raised blood pressure, compared with women (229; 35%). There was also a relationship between blood pressure values and proteinuria levels.

Implications of these findings

To date, this is the largest study to investigate chronic kidney disease in both men and women with Fabry disease. The results confirmed that a significant proportion of people with Fabry disease suffer moderate to severe kidney problems.

Proteinuria was common, and increased with decreasing kidney function [2]. This trend was previously reported not to be important in women with Fabry disease [6, 7]. However, a relationship between increased proteinuria and decreased kidney function now appears to exist in both men and women with Fabry disease, and it is possible that measuring proteinuria may help healthcare professionals to assess, and with appropriate treatment, slow kidney disease progression. Another important finding was that albuminuria was common among people with decreasing kidney function, and tended to appear before overt proteinuria developed. This means that albuminuria is a more useful early indicator of kidney disease than proteinuria.

This analysis also found that a number of people with Fabry disease had blood pressures higher than that recommended for those with chronic kidney disease. Of concern was that about half of men with stage 3–5 chronic kidney disease also had high blood pressure. Moreover, Ortiz and colleagues have more recently published another analysis of data from the Fabry Registry, focusing on people with end stage renal disease [8]. This found that about half of men with advanced chronic kidney disease who were receiving dialysis, had also suffered a serious clinical event associated with high blood pressure, such as a heart attack or stroke [8].

Although the Fabry Registry provides greater insight into the progression of Fabry disease and the spectrum of related complications, many patients, unfortunately, do not get diagnosed with Fabry disease until chronic kidney disease has progressed to an advanced stage. It is important to recognise these potentially dangerous complications as early as possible and thereby provide an opportunity for treatment and prevention of disease progression.

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Declaration of interest: The author is employed by Genzyme Corporation.

Supply situation for Replagal

Shire has been supplying enzyme replacement therapy for patients with Fabry disease in the UK since 2002 and following the Genzyme announcements of the problems at their manufacturing plant in July 2009, Shire have worked with the UK centres, the National Commissioning team and the MPS Society to make additional treatment available to meet patient treatment needs:

At the time of the initial Genzyme announcement it appeared that the problem would be only temporary and that no support from Shire would be needed. However, as the duration and extent of the ERT supply challenge quickly deepened in October 2009, Shire started receiving requests for additional use of Replagal from individual UK treatment centres and requests for general assurance on the ability to supply additional vials. The National Commissioning Group (NCG) co-ordinated various teleconference calls in which all parties participated and Shire was able to commit to meeting any and all requests for the use of Replagal. The co-ordination brought about by the NCG system meant that the UK had a more rapid response to meet patient needs than many other places in Europe.

This meant that Shire's manufacturing capacity had to step up to several challenges at once. In addition to the worldwide Fabrazyme shortage, there was a shortage of the enzyme for Gaucher Disease and a request was received from the Regulatory Authorities for Shire to make additional supplies available of its new ERT for Gaucher disease. This was totally unexpected and meant Shire had to advance its plans and manufacturing for this therapy by at least 18 months. At the same time manufacturing of Replagal was accelerated with teams working full shifts so as to ensure 24 hours a day manufacturing operations. The staff (mainly in the USA) worked through vacations and other personal commitments to ensure maximal output from the existing production capacity.

The additional demands on supply were not the only challenge this year for Shire, with the Icelandic volcanic ash cloud causing its own problems for refrigerated air freight transport of ERT vials into the UK. However arrangements were made to enable Shire to bring vials in by sea and land so that there were no interruptions to treatment due to unpredictable (and unpronounceable) volcano's!

Looking towards the future

Over the past three years, Shire has invested over $200million in new manufacturing capacity and technology to assure ongoing supplies of ERT. A new manufacturing facility in the USA has been constructed which implements a single use, disposable liner technology for the bioreactors used to produce the ERT. This new facility will also greatly increase the overall bioreactor capacity that Shire has to meet foreseeable future demand.

Shire's new 'Atlas' manufacturing plant in the USA

ENZYME REPLACEMENT THERAPY FOR FABRY
WHERE ARE WE NOW?

There is no doubt the shortage of Fabrazyme has presented a major challenge to the Fabry community as a whole, not least the patients. The initial request from Genzyme in July 2009 was to reduce Fabrazyme requirements for a period of a few months.

These requests called for concerted action by patients, carers, the Lysosomal Storage Disease teams at the various specialist centres across the UK and the Department of Health's National Commissioning Group (NCG) . A series of telephone conferences were held and a strategy was developed. Initially as it appeared that the shortage would be short-lived, we all agreed a dose holiday of one or two infusions over several months. By September 2009 it became obvious that the shortage of Fabrazyme was much more serious and unlikely to be resolved into 2010. At this point, many Fabry sufferers, who relied on Fabrazyme as their Enzyme Replacement Therapy (ERT), were being transferred to Replagal, an alternative ERT for Fabry disease. As the true extent of the Fabrazyme shortage only became known in a drip drip fashion, it has been incredibly difficult for doctors in respect of making treatment decisions in the best interests of their patients, and indeed for the patients themselves.

The Fabrazyme shortage continues and it now looks as if normal supplies of Fabrazyme will only be available towards the end of 2010 or early 2011. This is positive news for patients that have continued on Fabrazyme throughout the shortage and for those on alternative products who wish to transfer back to Fabrazyme. We do not underestimate the upheaval patients have felt. Many of you we know have transferred to Replagal and some of our members have enquired recently as to whether they have to change back to Fabrazyme. If you, or you acting for your child, do not wish to transfer back to Fabrazyme you should be having this discussion with your Fabry doctor or child's paediatrician. If there is no over-riding clinical reason given for transferring back to Fabrazyme, the decision as to which product you are on should be yours. It is always a good idea to ask your Fabry doctor to explain in lay terms his or her reasons for prescribing one ERT over the other and the clinical benefits to you or your child. **Christine Lavery** c.lavery@mpssociety.co.uk

European Medicines Agency updates treatment recommendations because of continued Fabrazyme shortage

Doctors advised to consider switching patients to alternative treatment

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) has been obliged to revise its previous recommendations on the use of Fabrazyme (agalsidase beta). This follows information from the manufacturer, Genzyme, stating that the current supply of Fabrazyme will not address the medical needs of the nearly 600 patients receiving Fabrazyme in Europe today. The CHMP is recommending that in situations where alternative treatment is available, no new patients should be started on Fabrazyme. For patients receiving a dose of Fabrazyme less than 1 mg/kg every other week, physicians should consider switching to an alternative treatment, such as Replagal.

These recommendations are temporary and do not change the currently approved product information for Fabrazyme.

The supply shortage of Fabrazyme began in June 2009 and was caused by a series of manufacturing problems at the production site in Allston Landing, in the United States of America. Because the current productivity at Allston Landing is still lower than expected, supply of Fabrazyme will not return to normal before the end of this year, according to Genzyme.

Fabrazyme is used to treat the rare, inherited enzyme-deficiency disorder, Fabry disease. Temporary treatment recommendations to manage patients relying on these medicines have been in place since the start of the manufacturing problems and have been regularly updated.

The CHMP remains concerned about the continued supply shortages of Genzyme's medicines. It is currently assessing proposals for improvement measures put in place by Genzyme to prevent similar manufacturing and quality problems in the future, and is closely monitoring the implementation of these measures. The Agency will make further updates as appropriate.

Amicus Phase 3 Fabry 011 Study: FACETS Study Update

The FACETS Study (AT1001-011) is the first Phase 3 clinical trial to study an oral investigational drug for Fabry disease. This global study to support U.S. FDA approval, is assessing AT1001 (miglastat hydrochloride), Amicus' pharmacological chaperone.

Pharmacological chaperones are orally administered, small molecule drugs designed to increase the activity of specific proteins. Chaperones selectively bind to and stabilise the target enzyme naturally produced by a person's cells. This leads to increased enzyme activity and a decrease in the accumulated substrate in affected tissues.

The FACETS Study is divided into two stages. In the first stage, participants will receive either placebo (a sugar pill) or study drug (AT1001) for 6 months. In Stage 2, all participants will receive AT1001 for an additional 6 months, with an optional 12-month treatment extension period offered to participants who complete both Stage 1 and Stage 2.

Currently, the FACETS Study is enrolling individuals with Fabry at more than 40 study sites globally. Males and females, ages 16 to 74, with confirmed AT1001-responsive GLA mutations and who have never received enzyme replacement therapy (ERT) or have not received ERT for 6 months before the first study visit, may be eligible for participation.

Enrollment for the FACETS Study is expected to conclude toward the end of 2010, so please contact a study site or Amicus Patient Advocacy if you or someone you care about may be interested. More detailed inclusion criteria and additional information is available by speaking to your healthcare provider or by calling toll-free at 1-888-55-FABRY, visiting the FACETS website at www.fabrystudy.com, or www.clinicaltrials.gov and searching for Amicus Fabry, or by emailing *clinicaltrials@amicustherapeutics.com*

Find a FACETS Study Site:
For the most current site information, please refer to **www.fabrystudy.com** or **www.clinicaltrials.gov** and search for Amicus Fabry.

N. America:
Cedars-Sinai Medical Center, Los Angeles, CA
University of CA School of Medicine, San Francisco, CA
University of Colorado, Aurora, CO
Emory University, Atlanta , GA
Children's Memorial Hospital, Chicago, IL
University of Kansas, Kansas City, KS
Massachusetts General Hospital, Boston, MA
Columbia University Hospital, New York, NY
Cincinnati Children's Hospital, Cincinnati, OH
Children's Hospital of Pittsburgh, Pittsburgh, PA
Baylor-Dallas, Dallas, TX
University of Utah, Salt Lake City, UT
O & O Alpan LLC, Springfield, VA
University of Washington, Seattle, WA
The Hospital for Sick Children, Toronto, Canada
Hôpital du Sacré-Coeur, Montreal, Canada

Africa:
Cairo University Hospital, Giza , Egypt
Morningside Clinic, Johannesburg, South Africa

Australia:
Women's & Children's Hospital, Adelaide, Australia
Royal Melbourne Hospital, Parkville, Australia

Europe:
U. Z. Antwerpen, Antwerpen, Belgium
Rigshospitalet, Copenhagen, Denmark
Royal Free Hospital, London, England
Hope Hospital, Salford, England
Hôpital Raymond Poincaré, Garches, France
Universitaetsklinikum Wuerzburg, Wuerzburg, Germany
Policlinico Universitario Agostino Gemelli, Roma, Italy
Academish Medisch Centrum, Amsterdam, The Netherlands
Instytut Kardiologii, Warszawa, Poland
Hospital Universitario Miguel Servet, Zaragoza, Spain
Fundacio Puigvert, Barcelona, Spain
Gazi University Medical Faculty, Ankara, Turkey

Middle East:
Shaare Zedek Medical Center, Jersusalem, Israel

S. America:
University of Austral, Buenos Aires, Argentina
Hospital Britanico, Capital Federal, Argentina
Hospital del Salvador, Santiago, Chile
Hospital de Clinicas de Porto Alegre, Porto Alegre, Brazil
Federal University de Sao Paolo, Sao Paulo, Brazil
Hospital das Clinicals de Faculdade de Medicina, Sao Paulo, Brazil

FACETS Website Goes Live

Amicus is pleased to announce the launch of a new website dedicated to the FACETS Study for Fabry disease. The site is now up and running and contains information for healthcare providers, persons with Fabry, and their friends and family. In addition to helping you learn more about the clinical trial, the website includes a toll-free number. Callers can speak with an independent person – not an Amicus employee - who can help connect them with study site staff who can answer their questions. **www.fabrystudy.com**

Clinical Trial of Children with MPS IIIA (Sanfilippo A Disease)

A clinical trial has started at the Royal Manchester Children's Hospital (RMCH) involvingchildren diagnosed with MPSIIIA. This Clinical Trial is a Phase I/II Safety, Tolerability, Ascending Dose and Dose Frequency Study of Recombinant Human Heparan N-sulfatase (rhHNS) Intrathecal Administration via an Intrathecal Drug Delivery Device in Patients With Sanfilippo Syndrome Type A (MPSIIIA) sponsored by Shire Human Genetic Therapies Inc. The principal investigator is Dr Simon Jones, Consultant Paediatrician, Royal Manchester Children's Hospital.

This trial has been reviewed by the local Ethics Committee to check that the research work the research team at the RMCH are undertaking is sound and abides by all the rules for carrying out research in children. The Ethics Committee have approved this clinical trial.

If you are interested in receiving more detailed information about this study, and/or are interested in your child taking part in this study, please contact Dr Simon Jones or Prof Ed Wraith, or Research Nurses Kathryn McBride and Fiona Heap:

Dr. Simon Jones, Consultant Paediatrician
Prof. Ed Wraith, Consultant Paediatrician
Phone: 0161 701 2137/8
Fax: 0161 701 2303
Email: simon.jones@cmft.nhs.uk
Email: ed.wraith@cmft.nhs.uk

Kathryn McBride
Senior Paediatric Clinical Research Nurse
Phone: 0161 906 7500
Fax: 0161 906 7507
Email: kathryn.mcbride@wtcrf.nhs.uk

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Email us at
fundraising@mpssociety.co.uk
visit **www.mpssociety.co.uk** or
phone **0845 389 9901**

The Royal British Legion

We have four Poppy Break Centres for those who are recovering from an illness, bereavement or other life affecting events. The aim is to provide a comfortable and enjoyable Break.

Those eligible for a Poppy Break are serving or ex-Service men or women or their dependants who are in genuine need of a break.

Our 4 Poppy Break Centres are located in prime locations around the country.

We also provide two additional services, introduced in 2008, for Family Holidays & Adventure Breaks for young people.

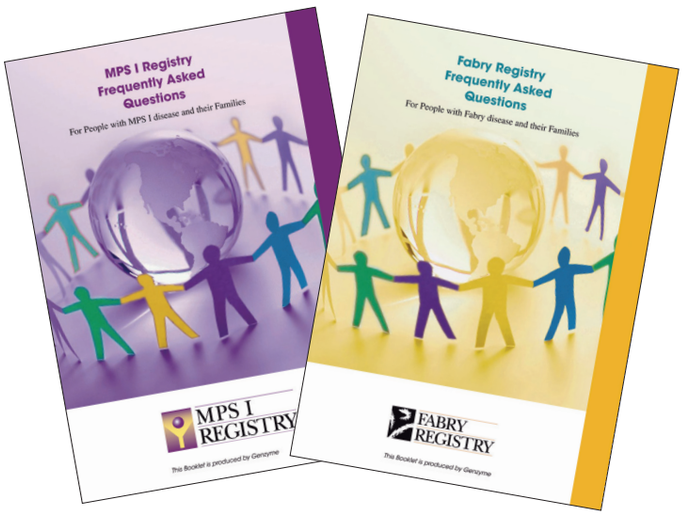
For more information please phone the MPS office on **0845 389 9901** or visit **www.britishlegion.org.uk/can-we-help/poppy-breaks**

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

It is vital that the MPS Society has sufficient funding to be able to look forward to the future with confidence. One way in which you can support the Society achieve its long term objectives is to include the Society when drawing up your Will.

For more information please contact us for our new **Leaving a Legacy leaflet** fundraising@mpssociety.co.uk or visit **www.mpssociety.co.uk** for more information

MPS I and Fabry Registries Frequently Asked Questions Booklets
– a resource for individuals with MPS I or Fabry disease, and their families



This year sees the launch of two Frequently Asked Questions (FAQ) booklets designed to answer all of the common questions posed by patients who are (or are considering) participating in the MPS I or Fabry Registries. The aim of these booklets is to inform individuals about the Registries, thereby allowing them to make an informed decision about whether they would like to participate or not. The FAQ booklets can be used to assist patient-doctor discussions during patient authorisation (or 'informed consent'), but also as a resource throughout participation to help patients explain to friends and family what taking part in the Registries means.

These booklets tackle the broader subjects of what disease registries are and why they are an important tool for research into rare diseases, as well as explaining how the Registries work and what to expect as a participant. Some example topics from the FAQ booklets (abridged):

What is a disease registry?
A disease registry is a database that collects information from a wide population of individuals with a specific, often rare, disease. By definition, each rare disease only has a small number of patients, making it difficult to collect information about the signs and symptoms

What are the MPS I and Fabry Registries?
The Registries are large, ongoing observational databases sponsored by Genzyme, with the aim of monitoring the long-term natural course of the diseases and treatment outcomes. The MPS I and Fabry Registries collect data from patients worldwide and will be maintained over a long period of time, thereby providing information that would ordinarily be difficult to obtain because the populations are too small

Who can participate in the MPS I and Fabry Registries?
The Registries are open to all people with MPS I or Fabry disease, respectively, regardless of treatment status or treatment choice

Why is my participation important?
For a registry to be effective, it needs to include as many people as possible to improve the accuracy of the conclusions drawn from the data. Data from patients who are either treated or untreated are equally important to enable research into the disease and long-term effects of treatment

Who will submit the data?
Clinical information is collected by the medical team during regular clinic visits and then submitted to the Registry, usually by a nurse or data entry specialist

How can I see my progress?
Once enough data are entered, a summarised report of your clinical progression is available. You may wish to ask your doctor if you would like to obtain and understand this overview, which may provide you with additional insight into your medical condition

Should I take part in more than one registry?
Registries can collect different kinds of information, and enable data from a large number of people to be combined so that trends in disease progression, management, and treatment outcomes can be identified. You may wish to discuss the practicality of taking part in more than one registry with your doctor

Copies of the MPS I and Fabry Registries FAQ booklets are available to all patients from the LSD treatment centres and from the MPS Society.
mps@mpssociety.co.uk

Anna Bishop-Bailey PhD
UK Lysosomal Storage Disorders (LSD) Registry Coordinator, Genzyme

The Public Health Action Support Team

In North East London there are 660,000 people aged between 15 and 40 years. Currently we do not know how many of these have specialist palliative care needs. Children's palliative care services are well established – though there is always room for improvement – as is specialist palliative care provision for older people. But in the last two decades a new group has been recognised, children who previously would have died young now living into early adulthood, and young adults in their 20s and 30s who contract serious illness needing palliative care.

PHAST is carrying out a needs assessment for this transition age group in North East London, with a project managed by Richard House Children's Hospice, funded by the Samuel Sebba Trust.

The needs assessment will determine expected numbers of young people with conditions that might require specialist palliative care and also aims to find out how many young people are currently getting these services. Some young people moving out of children's care have found little to meet their needs.

But what services are needed and wanted?

What should young people's specialist palliative care look like?

The project has already engaged with a number of young people in their late teens who are 'transitioning' to adult services to seek their views. There is now the opportunity to develop and shape a new approach to specialist palliative care for young adults. Some young people have had regular contact with children's hospices and have experienced respite care, admission to acute hospitals, community care by nurse teams and a range of other support. Adult specialist palliative care tends to consist of symptom control interventions and intensive support at the end of life either at home or in a hospice and usually for a much older age group. Young people with a condition they have always lived with need more all round support and their perspective is towards living, not dying.

PHAST would be very grateful to hear the views of MPS Society members from a wider geographical area. What has your experience been of 'transition'? We would especially like to hear of good practice, what has worked well. Could you send your story? Of describe for us what would really help in replacing the supportive environment you have known in a children's hospice. Even more, we would like to hear from young people and their families (especially in NE London, but other places too) who have not had children's hospice support. Why has this been and what could help?

Margaret Simmons BSc MHM FFPH
Public Health Specialist and PHAST Associate

52 Fairford Avenue
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LU2 7ER
T: 01582 420140
Mob: 07974 692444
E: Margaret.simons@clara.co.uk

If you wish to forward your views please contact Margaret directly or send in to MPS and we will forward.

Homes with Heart

We provide an easy way for holiday home owners to put their spare weeks to good use, helping families suffering from a life-limiting illness have a holiday.

What we do
We launched in June 2008 and have arranged over 60 successful holidays since that time. We manage The Directory, which is a register of all holiday homes where the owner is happy to 'bank' some spare time to families desperately in need of a break. Healthcare professionals know that we hold this list and can 'withdraw' a week at a time for those young families they know would benefit most - families where parents or children are terminally ill or bereaved.

How it works
The onus is on healthcare professionals to refer a family whom they have known for more than a year and to support their need for a break. It is often the quieter families, who would never ask, who need some time away and only their care team have the experience to see that. Individuals can complete the form but they must attach a supporting letter from a GP or care worker for their application to be considered.

Download a Holiday Respite Application Form online at **www.homeswithheart.co.uk**
Tel: 01372 842751
Email: info@homeswithheart.co.uk

Silk cushions solve frizzy hair challenge for Jade!

Hello! My name is Deby McAfee and the mother of Jade who is 12 years old and in stage 3 of Sanfilippo Disease.

I'm sure like many other MPS parents I find working with Jade's hair a real challenge, as it is so coarse and frizzy and now because she is lying down more it has become very split and broken at the back of her head. She is on fish oils for her hair, skin and joints. This has had a big impact on her joint pain and I would give it to her via her peg with her milk feed.

While we were in a hospice for our summer break a lady gave me a magazine to read and there was a advert on silk pillows and the benefits they have on your skin and hair. I thought that was really interesting and I checked it out on the internet.

The pillow they had advertised was £45.00 plus postage. I thought that was quite expensive but it would be worth it if it works. Anyhow, whilst on Google I came across a super site called Cliffton Silk Gifts. I rang the lady and we had a lovely chat. I asked if she could post a pillow case to me first class on the Monday morning and it arrived with me on the Tuesday.

It's great and I'm so impressed. In the mornings there are no knots or tugging to get Jade's hair brushed and no tears. Also her hair is in much better condition already and she is sleeping really well.

I would really recommend trying it out. At £22 Free UK delivery they are so worth it!

Email **enquiries@silkgifts.co.uk**
or contact Sue at CLIFFTON SILK GIFTS
201-201 Stubbington Avenue
Portsmouth
Hampshire PO2OJW
Tel: 02392 655518

Jade (MPS III)

INFORMATION RESOURCES

The MPS Office has some extra copies of the following publications. If you would like a copy please send a large £2 stamped addressed envelope to Sue Cotterell at the MPS Society's address at MPS House and the booklets will be dispatched on a first come first served basis.

What's Missing
This 6 page booklet provides a deeper understanding of Misfolded Enzymes and Lysosomal Storage Diseases. It is published by Amicus Therapeutics and in full colour and clear illustrations simply explains the subject for the lay person.

It's All in Your Hands
This double DVD has been developed by Birmingham Children's Hospital to explain carpal tunnel syndrome surgery for MPS and the correlation and the correlation between carpal tunnel and MPS diseases. Viewing is not for the squeamish or weak hearted but most informative if you or your child are dealing with carpal tunnel syndrome and facing surgery.

Working Party on Sleep Physiology and Respiratory Control Disorders in Childhood – Executive Summary September 2009
This 36 page Executive Summary conducted by the Royal College of Paediatrics and Child Health sets out the Standards of Services for Children with Disorders of Sleep Physiology. The report which includes the Mucopolysaccharidoses presents evidence-based recommendations for the diagnosis and management of disorders of sleep physiology and respiratory control in children, and the organisation of such services nationally in the UK.

Buggy wanted!

We have had a request from one of our MPS member families asking if you have a buggy your child no longer needs that could be donated to a child with a progressive disease in Pakistan. The boy concerned weighs 20 kilos and has very long legs. He is 4 feet 4 inches tall.

Until now this boy has received the discarded buggies of the MPS family's daughter but sadly their daughter has died and the boy has grown out of his current buggy.

If you can help please call MPS on **0845 389 9901** or email **c.lavery@mpssociety.co.uk**. The MPS family will organise any buggy's transportation to Pakistan.

Resources from the Challenging Behaviour Foundation

New resource: **A guide for advocates** – supporting people with learning disabilities who are described as having challenging behaviour

The Challenging Behaviour Foundation has produced a new resource for advocates in England and Wales supporting people with learning disabilities who are described as having challenging behaviour.

Characteristics of good support are identified, with suggested questions for advocates to ask around personalisation, activities and opportunities, staff training and communication skills. The guide also looks at issues such as the use of medication, physical intervention, barriers and seclusion, sectioning under the mental health act and alleged offending. Key 'at a glance' bullet points identify 'what you may encounter' and 'what you should know' – a very practical tool for both professional advocates and family carers advocating on behalf of their family member.

The Challenging Behaviour Foundation specialises in providing information and support to those caring for individuals with severe learning disabilities, however much of the information in the guide will also be relevant to people described as having challenging behaviour who have mild or moderate learning disabilities.

Cost: £16.00 (includes postage and packing in the UK only). Free to family carers. To order please contact:
The Challenging Behaviour Foundation
Tel. 01634 838739
Email info@thecbf.org.uk
www.challengingbehaviour.org.uk
General Enquiries: Tel. 01634 838739
Family Support Worker: Tel. 0845 602 7885 (individual telephone support for families at the cost of a local call)

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

New Challenging Behaviour charter launched

The Challenging Behaviour – National Strategy Group has launched a charter to promote the human rights of individuals with learning disabilities who are perceived as challenging.

Up to 27,000 people with learning disabilities in the UK may have been given a label of challenging behavior, resulting in this group of people being – stigmatised and socially excluded denied the right to ordinary lives in the community, to education, recreation and employment placed in institutional settings a long way from home and families.

The label challenging behaviour, has become misused over time. Rather than being used as a term to encourage carers and professionals to understand the underlying reasons for a person's behaviour, 'challenging behaviour' has been used as a diagnostic label, viewed as being intrinsic to the person.

The Challenging Behaviour – National Strategy Group want people (and organisations) to sign up to the charter to register their support for the principles it contains and to commit to action to improve the lives of children and adults who are labelled as challenging. We need as many people as possible to support us, so please ask your friends and family to sign up too.

To read the charter, visit: www.challengingbehaviour.org.uk/StrategyGroup/Charter

What is the Challenging Behaviour – National Strategy Group?
The Challenging Behaviour – National Strategy Group (CB-NSG) was launched on November 7th 2008. The CB-NSG is a key national group to address the needs of children, young people and adults with learning disabilities whose behaviour is perceived as challenging.

Members of the CB-NSG include family carers, representatives from the Department of Health, Royal College of Psychiatrists, British Psychological Society, Royal College of GP's, NHS Trusts, researchers, service providers and a range of practitioners, regulators, commissioners and third sector representatives. The group is action and outcome focused and comes together twice a year to monitor progress, share best practice and develop coordinated action plans.

What is challenging behaviour?
"Behaviour can be described as challenging when it is of such an intensity, frequency, or duration as to threaten the quality of life and/or the physical safety of the individual or others and it is likely to lead to responses that are restrictive, aversive or result in exclusion." (Challenging behaviour – a unified approach; RCPsych, BPS, RCSLT, 2007)

Challenging behaviour is things like hitting your own head against a wall, pulling curtains down or pulling someone's hair. Often people do this because they cannot communicate with words and they have little or no choice and control over what is happening to them.

How do I find out more?
To find out more about the Challenging Behaviour National Strategy Group, please refer to 'All change' the Summer issue of 'Challenge' today. This issue focuses on the work of the National Strategy Group and includes articles from Dr Roger Banks (Consultant in the Psychiatry of Learning Disabilities), Jackie Edwards (Family carer) and Bob Tindall (United Response).

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