

Celebrating 35 years
of the MPS Society

MPS Society conference

on paediatric and adult perspectives in management and
treatment of mucopolysaccharide, fabry and related diseases

7-9 July 2017
Coventry, UK



Welcome

In 2017 we celebrate the 35th anniversary of the Society for Mucopolysaccharide Diseases – and what an amazing 35 years they have been!

In 1982, at its conception, there were just 40 MPS families in the MPS Society. At our first MPS family weekend conference in September 1983 in Walsall, Birmingham, 50 MPS families participated, hungry for information.

For the next 20 years the only treatment available that changed the course of the disease was Bone Marrow Transplant (BMT) for MPS I Hurler so the focus was inevitably on care and research whilst scientists and clinicians worked in earnest to bring about disease changing breakthroughs in treatment.

In 2003 Enzyme Replacement Therapy (ERT) for MPS I was approved in Europe opening the gate to a remarkable 15 years in drug development for MPS, Fabry and related diseases.

Our MPS weekend conference programmes have, and continue, to highlight the latest developments in clinical management and treatment ensuring those at the coal face, our members, have the most up to date information available as well as the opportunity to network with experts in the MPS community including clinicians and healthcare professionals, scientists and researchers, all under one roof over one weekend.

The 2017 weekend conference in Coventry offers a fitting opportunity to reflect upon and celebrate the successes of the MPS Society over the past 35 years.

Christine Lavery, MBE

The MPS Society are holding a weekend conference, from Friday 7 July to Sunday 9 July 2017. This unique conference is dedicated to sharing the most up to date information available on the clinical management and treatment of MPS, Fabry and related diseases as well as clinical trials and research.

Our Saturday programme offers three separate conferences running in parallel and focusing on:

- **Conference A** – MPS I, MPS II (not progressive CNS), MPS IVA, MPS VI, LAL D (late onset), ML III and Mannosidosis.
- **Conference B** – MPS II (progressive CNS), MPS IIIA, B, C, D, LAL D (infantile) and rare related diseases.
- **Conference C** – Fabry disease.

As well as the professional presentations each conference will contain a variety of talks reflecting the personal experiences of members affected by these diseases. The programme also offers the unique opportunity for all those participating to meet and share experiences in a relaxed environment.

This conference is open to all those living with or affected by MPS, Fabry or a related diseases as well as health, social care and educational professionals. This is an unmissable event for those personally or professionally involved in caring for a child or adult with MPS, Fabry or a related disease.

To enable parents and carers to make the most of the weekend conference affected children and their siblings, aged 17 years and under along with affected vulnerable adults will have their own social programme packed full of fun and stimulating activities and outings.

About the conference

The venue

The conference venue is the Hilton Hotel Coventry, ideally located just off Junction 2 of the M6/M69 interchange, only 20 minutes drive from the NEC Birmingham. The hotel is open plan, spacious and spread over four floors with the conference facilities on the ground and first floor. The restaurant and some of the bedrooms are on the ground floor.

Hilton Hotel rooms will be given as a priority to families with children. Ground floor bedrooms will be allocated on a first come, first serve basis with priority being given to those with wheelchair needs although there are two lifts to the other three floors.

The hotel benefits from its own indoor heated swimming pool. Other leisure facilities include the Livingwell Health Club with an air conditioned gym, sauna and steam room. Beauty treatments and massages are available by appointment. These facilities will be available to all guests over the course of the weekend (subject to a cost). Guests arriving on the Friday will be able to use the hotel facilities, however bedrooms are not guaranteed to be ready until 3pm.

If the bedrooms at the Hilton Hotel are oversubscribed, alternative accommodation has been booked with the Premier Inn which is based very close to the Hilton Hotel, with a shuttle service running between, so it is vitally important that MPS families in particular book early.

Accommodation at the Hilton Hotel is on a first come first served basis, with priority given to families with children.



Hilton Hotel
Paradise Way
Walsgrave Triangle
Coventry
CV2 2ST
Tel: 0247 660 3000
www.coventryhilton.com

Premier Inn
Gielgud Way
Cross Point Business
Park
Coventry
Tel: 0871 527 8266
www.premierinn.com



**The last chance to
book your place
for the conference is
Friday 19 May 2017**

Please note that the MPS Society reserves the right to alter the programme at any time

Children and vulnerable adults' programme

Our children and vulnerable adult's programme is possible thanks to our wonderful volunteers

We are planning an exciting childcare programme for all children under 18 years old as they are not permitted into the conference. We hope this will allow parents and carers to attend the conference in a relaxed manner, knowing that their children are safely cared for and entertained.

Who it's for

Children with MPS or related diseases and their siblings aged 17 and under will have their own social programme. This programme will also accommodate young adults over the age of 18 years who are unable to benefit from the conference due to their neurological or high-care needs. Young adults over the age of 18 years who wish to be included in the childcare programme may do so by prior agreement of the Society.

We are planning a programme that will be suitable to all the children and young people attending, however, it may not be possible for us to take some individuals on excursions. This might include those who are no longer mobile or able to weight bear and those with complex needs such as requiring gastrostomy feeds, suctioning and who require complex care when out. We will assess everyone on an individual basis and our decisions will be dependent on whether we are able to secure volunteers who have the experience to provide the level of care required.

We will be providing on-site care for children under the age of 3 years and children and young adults who are unable to participate in any outings or the conference. This facility is spread over a number of rooms and will include a variety of activity-based rooms and a dedicated

care space. Please be aware that we are bound by the constraints of the hotel and facilities may be limited and that there may also be large differences in age and needs will be wide ranging.

Evening care and entertainment

As well as the daytime programme, our volunteers will provide care on the Saturday evening to allow parents and carers to attend the gala dinner. Please note at this stage we cannot guarantee we will be able to provide care in individual bedrooms and we may have to provide some form of entertainment within the hotel.

About our volunteers

Our children and vulnerable adult's programme is possible thanks to our wonderful volunteers who will be at the conference throughout the weekend. Each volunteer completes an application, two references and undergoes a DBS check. Each volunteer is allocated a child, group of children or a vulnerable adult and given all the information needed about the needs of the individuals in their care. We make sure we consult with parents or carers about their child/children's needs and a care plan will be drawn up where needed. Volunteers receive specific training on moving and handling, advice on safeguarding children and vulnerable adults and volunteer conduct.

The Society will endeavour to meet each individual need and childcare volunteers will be allocated on a first come, first served basis. If your child has complex care needs please get in touch before booking to discuss needs and resources required.



Conference A

Focus on MPS I, II (not progressive CNS), IVA, VI, LAL D (late onset), ML III and Mucopolysaccharidosis

Chair: Alison Wilson and Bob Stevens

Clinical management and best care

- 9.00 Welcome
- 9.10 Overview on MPS diseases – James Davison
- 9.30 Orthopaedic management of the spine – tbc
- 9.50 Meeting the orthopaedic needs from the hips down (knees/hips/ankles) – Elaine Robinson
- 10.10 Carpal tunnel and the hands – Andrea Jester
- 10.30 Respiratory complications in MPS – Stuart Wilkinson
- 10.45 **Comfort break**
- 11.00 Cardiovascular disease in MPS diseases – Peter Woolfson
- 11.15 Dental care in MPS – Eleanor McGovern
- 11.30 Ophthalmic presentations in MPS conditions – Jane Ashworth
- 11.45 Meeting the psychological challenges of young people with MPS conditions
Stewart Rust
- 12.00 Outcomes from MPS I behaviour study – Annukka Lehtonen
- 12.15 Ask the experts
- 12.45 **Lunch**

Chair: Paul Moody and Stewart Rust

Advocacy

- 13.45 Education Healthcare Plan explained – Teresa Jeffery
- 13.55 Choices and achievements moving into Further Education – Aisha and Asma Seedat
- 14.10 How benefits and access to services change when individuals become an adult – Bob Stevens
- 14.25 Health and Wellbeing: quality of life experiences of an MPS I sufferer – Aaryanna Lever
- 14.40 Practical advice and outcomes from UK pregnancy project – Alison Wilson
- 15.00 **Comfort break**

Treatment

- 15.15 How self-infusing benefits our family – Claire Stevens
- 15.25 How we arrived at the managed access programme and future challenges – Charlotte Roberts
- 15.45 Overview of current treatments – Anupam Chakrapani
- 16.05 How Natural History studies help us to better understand disease progression – Simon Jones
- 16.25 Role of homecare nurses – Rene Sonders
- 16.40 The fight to get Vimizim approved and its impact – Katy Brown
- 16.50 Question and answer session
- 17.15 Close

Conference B

Focus on MPS II progressive CNS, MPS III, MLD, LAL D (infantile) and rare related Diseases

Chair: Brian Bigger and Jessica Kafzas

Clinical management and best care

- 9.00 Welcome
- 9.10 Overview of MPS and related Diseases – Julian Raiman
- 9.30 Meeting the orthopaedic needs in individuals with neurological decline – Michelle Wood
- 9.50 Respiratory complications and interventions – Chris Hendriksz
- 10.10 Cardiac manifestation in MPS III and MPS II – Ashish Chikermane
- 10.30 Disease manifestations in conditions with late onset – Chris Hendriksz
- 10.45 **Comfort break**
- 11.00 Nutritional support in MPS and related diseases (LAL D/MLD) – Fiona White
- 11.15 Seizure management – Sanjeev Rajakulendran
- 11.30 How we measure cognitive function in individuals with progressive neurological disease – Julie Eisengart
- 11.45 Behaviour management techniques – Shauna Kearney
- 12.00 How we manage our child's behaviour: useful hints and tips – Karen Robinson
- 12.15 Swallowing difficulties and MPS – Jo Marks
- 12.45 Ask the Experts
- 13.00 **Lunch**

Chair: Judith Evans and Chris Hendriksz

Advocacy

- 13.45 Sleep disturbance and how to manage as a parent/carer – Stewart Rust
- 13.55 Do children with MPS III have symptoms of Autistic Spectrum Disorder (ASD) – Dougal Hare
- 14.10 Continuing healthcare assessments explained – Louise Cleary
- 14.25 Mental capacity and guardianship – Sophie Thomas and Peter Hawkins
- 14.40 Planning for the future: getting the right equipment and adaptations in place – Charlotte Starling
- 15.00 **Comfort break**

Clinical trials leading to market access and reimbursement

- 15.15 Overview of current treatments – Maureen Cleary
- 15.35 How natural history studies help us to better understand disease progression – Saikat Santra
- 15.55 Gene therapy for MPS conditions with neurological decline – Joseph Muenzer
- 16.25 Understanding the role of the Patient Access to Clinical Trials and Treatments team – Christine Lavery
- 16.35 Families experience of being enrolled on a clinical trial – Daniella Vandeppeer
- 16.50 Supporting multiple trials for MPS III – Joanne Goodman
- 17.00 Question and answer session
- 17.15 Close

Conference C

Focus on Fabry

Chair: Atul Mehta and Uma Ramaswami

Best practice clinical management

- 9.00 Welcome – Atul Mehta
9.10 Overview of Fabry disease and current understanding – Derralynn Hughes
9.30 Current understanding of cardiac involvement in Fabry disease – Perry Elliot
9.50 The role of dialysis and transplantation in Fabry Disease – Gere Sunder-Plassmann
10.10 Mutations analysis what we know and how this can predict disease severity – Derralynn Hughes
10.30 Fabry manifestations in the eye: what do we know? – Jane Ashworth
10.50 Overview of current treatment options – Atul Mehta
11.10 Comfort break
11.30 Do females with Fabry Disease frequently have major organ involvement? – Alberto Ortiz
11.40 Pain in Fabry Disease and its management – Max Hilz
12.00 Life and living with Fabry – Naomi Carter
12.20 Fabry and planning a family: what are my options? – Alison Wilson
12.35 Ask the Experts
13.00 Lunch
13.50 Challenges in transitioning from DLA to PIP – Sophie Thomas
14.05 How to answer questions when having a medical assessment for benefits – Les Hilliard and Sophie Thomas
14.25 Value of joint family clinics – Uma Ramaswami
14.45 Self-infusing works for me: a patient story – Kelly Garrett
15.00 Comfort break
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15.15–16.45 Group sessions

Session 1 (15.15–16.00) Toni Ellerton and Alison Wilson

Group 1: Pain management – Derralynn Hughes and Max Hilz

Group 2: Understanding the psychological needs of Fabry Disease? – Uma Ramaswami

Session 2 (16.00–16.45) Toni Ellerton and Alison Wilson

Group 1: Symptom management/sharing experiences – Uma Ramaswami

Group 2: Pain management – Derralynn Hughes and Max Hilz

- 16.45 Feedback from sessions
17.00 Close

Conference A

Therapies – MPS I/MPS II/MPS IV/MPS VI/LAL D late onset

Chair: Bryan Winchester and Paul Moody

- 9.00 Overview of ERT: where we are today – Bryan Winchester
- 9.15 Intrathecal ERT for MPS II – Suresh Vijay
- 9.30 ERT for MPS IV: where we are 18 months on – Chris Hendriksz and Alexandra Morrison
- 9.50 HSCT gene therapy in MPS conditions – Su Han
- 10.00 Outcomes from ERT trials for MPS I – Paul Orchard
- 10.20 Introduction to gene therapy – Joseph Muenzer
- 10.45 **Break**
- 11.00 Intra-cerebral gene therapy for MPS I and MPS II – Simon Jones
- 11.10 Intravascular gene therapy for MPS VI – Generoso Andria
- 11.20 Intravenous gene therapy for MPS I and MPS II – Simon Jones
- 11.30 ERT for LAL D late onset – Reena Sharma
- 11.50 Emerging treatment for Alpha Mannosidosis – Alex Broomfield
- 12.00 Pentosan Polysulfate (PPS) for treating inflammatory disease in MPS disorders – Julia Hennermann
- 12.10 Panel questions
- 12.45 End

Conference B

Therapies – MPS III/MLD/LAL D (infantile)

Chair: Maureen Cleary and Jessica Kafizas

- 9.00 Overview of current and emerging treatments – Brian Bigger
- 9.20 Outcomes from clinical trials for infants with LAL D – Arunabha Ghosh
- 9.40 Learnings from the Genestein trial – Brian Bigger
- 10.00 HSCT gene therapy in MPS III – Brian Bigger
- 10.15 Intracerebroventricular infusion for MPS III B – Maureen Cleary
- 10.35 Intravenous ERT for MPS III B – Suresh Vijay
- 10.55 **Break**
- 11.10 What is the difference between in vivo and ex vivo gene therapy – Simon Heales
- 11.30 Intracerebral gene therapy for MPS III and MLD – Maureen Cleary
- 12.10 Panel questions
- 12.30 End

Conference C

Fabry

Chair: Charlotte Roberts and Derralynn Hughes

- 10.00 Learnings and next steps in regards to ERT – Derralynn Hughes
- 10.20 Chaperone treatment for patients aged 16 years and over with an amenable mutation – Derralynn Hughes
- 10.40 New and emerging treatments for Fabry disease – Ana Jovanovic
- 11.00 **Break**
- 11.15 The patient's experience of Fabry Disease and treatment – Jacqueline Adam
- 11.35 Receiving a diagnosis in hindsight what would I change – Nicola Carnall
- 11.55 What is a managed access agreement and what does this mean? – Charlotte Roberts
- 12.15 Discussion
- 12.30 End

Booking form

Individual and family booking for UK members

(choose either the residential OR non-residential option)

| Residential Includes Friday and Saturday night accommodation, Friday dinner, Saturday breakfast and lunch, Saturday dinner, Sunday breakfast and lunch and Saturday and Sunday conference. | price per room (unless stated) | quantity | | subtotal |
|--|---------------------------------------|-----------------|------|-----------------|
| Single room For members aged 18+ attending the weekend alone. This cannot be offered at this price to additional adults who are accompanying other families. | £160 | | | |
| Double/Twin room Maximum two persons, must include one adult (Double room: one double bed, Twin room: one double and one foldaway bed) | £230 | double | twin | |
| Family room For two adults, MPS sufferers and siblings under 18 years of age | £290 | | | |
| An additional adult sharing a family room (price per additional person) | £210 | | | |

| Non-residential options Please note that the Saturday and Sunday conferences are for adults only. We are unable to accommodate children on the childcare programme if you have not booked for the residential weekend. | | price per person | quantity | subtotal |
|--|--------------------|-------------------------|-----------------|-----------------|
| Saturday conference only with lunch (18 years or over) | MPS/Fabry sufferer | £18 | | |
| | Adult | £34 | | |
| Saturday conference only with lunch and gala dinner (18 years or over) | | £70 | | |
| Sunday conference only with lunch (18 years or over) | MPS/Fabry sufferer | £12 | | |
| | Adult | £20 | | |

| | | | | |
|--------------|--|--|--|--|
| Total | | | | |
|--------------|--|--|--|--|

Professionals, non-members and international delegates

(choose either the residential OR non-residential option)

| Residential Includes Friday and Saturday night accommodation, Friday dinner, Saturday breakfast and lunch, Saturday dinner, Sunday breakfast and lunch and Saturday and Sunday conference. | price per person | quantity | | subtotal |
|--|-------------------------|-----------------|------|-----------------|
| Single room | £280 | | | |
| Double/Twin room Two people sharing (Double room: one double bed, Twin room: one double and one foldaway bed) | £255 | double | twin | |

| Non-residential options Please note that Saturday and Sunday meetings are for adults only and we are unable to accommodate children on the childcare programme if you have not booked for the residential weekend. | price per person | quantity | subtotal |
|--|-------------------------|-----------------|-----------------|
| Saturday conference only with lunch | £85 | | |
| Saturday conference only with lunch and gala dinner | £120 | | |
| Sunday conference only with lunch | £50 | | |

| | | | | |
|--------------|--|--|--|--|
| Total | | | | |
|--------------|--|--|--|--|

Details of main contact

Name:

Organisation (if applicable):

Address:

Post code:

Phone:

Email:

Delegate information (for everyone in your party including main contact)

| First name | Surname | Special dietary requirements | Sat conf* | Sun conf* | Wheel chair | DOB (if under 17) | MPS/Fabry |
|------------|---------|------------------------------|--------------------------|--------------------------|--------------------------|-------------------|--------------------------|
| | | | <input type="checkbox"/> | <input type="checkbox"/> | <input type="checkbox"/> | / / | <input type="checkbox"/> |
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| | | | <input type="checkbox"/> | <input type="checkbox"/> | <input type="checkbox"/> | / / | <input type="checkbox"/> |

*Please indicate which conference you would like to attend using **A, B** or **C**

Saturday

A: Focus on MPS I, II (not progressive CNS), IVA, VI, LAL D (late onset), ML III and Mannosidosis

B: Focus on MPS II progressive CNS, MPS III, MLD, LAL D (infantile) and rare related Diseases

C: Focus on Fabry

Sunday

A: Therapies – MPS I/MPS II/MPS IV/MPS VI/LAL D late onset

B: Therapies – MPS III/MLD/LAL D (infant)

C: Fabry

Mindfulness workshop

At this conference we are offering a workshop on mindfulness and how it can help with the emotional journey with an MPS child or as an adult MPS sufferer. This is included in the residential package and is open to adults and young adults aged 16 and over.

I would like to attend the mindfulness workshop for parents and professionals on Friday at 4.30pm

I would like to attend the mindfulness workshop and dinner for young adults on Friday at 7.00pm

Contact preferences

We want to keep you up to date with our news, events, campaigns and other things we're proud of (including the MPS Magazine) but only with your permission. We promise to keep your data safe and never ever sell it on. If you're happy with this please tell us how you would like to be contacted by ticking all the relevant boxes below.

by email by mobile/text by telephone by post

To change these options at any time call us on [0345 389 9901](tel:03453899901) or email mps@mpssociety.org.uk or find out more about Our Donor & Fundraiser Promise at www.mpssociety.org.uk/about/promise

Payment

Name and address of account holder (if different to contact address)

Name: _____

Organisation (if applicable): _____

Address: _____

_____ Post code: _____

Phone:: _____

Payment method

Total to pay: £

- Pay by cheque (made payable to: MPS Society)
- Invoice hospital or institution (professionals only)
- Pay by credit or debit card (complete form below) Diners/Discover, JCB, Maestro, Mastercard, Visa, and Visa Electron accepted

Payment by card (*required information)

Name on card*: _____

Address of card holder*: _____

_____ Post code*: _____

Card number*: _____

Issue number: _____ Valid from: / _____ Expiry date*: / _____ Security code*: _____

Please complete the booking form and payment details then tear off the last four pages of this booklet and return to us at:

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

Fax: 0345 389 9902

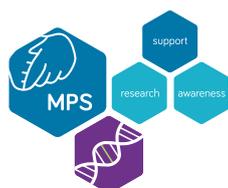
Email: mps@mpssociety.org.uk

For any queries call 0345 389 9901

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Society for Mucopolysaccharide Diseases

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