



Society for
Mucopolysaccharide
Diseases

Expert Meeting
on Sanfilippo Disease
(MPS III)

Friday 27 - Saturday 28 August 2010
Hilton Northampton

PROGRAMME

Expert Meeting on Sanfilippo Disease

This expert meeting on Sanfilippo disease offers individuals, parents, partners, carers and professionals the opportunity to hear state of the art talks on the clinical management of those affected by MPS III and members' personal experiences, as well as finding out more about the latest research developments and potential therapies.

The expert meeting programme offers the opportunity to meet and talk informally with professionals, and for individuals, parents, partners and carers to share their own experiences with others.

Children and vulnerable adults affected by Sanfilippo disease and their siblings aged 17 and under will have their own social programme, which will allow them to have fun whilst giving them the opportunity to talk to and make friends with others in similar circumstances.

The expert meeting is spread over two days from Friday to Saturday and will be the largest gathering of Sanfilippo sufferers, their families and professionals ever. Doctors and researchers will also be present.

Children and Vulnerable Adults Programme

No children under 18 years are permitted into the expert meeting. There will be a children and vulnerable adults activity programme arranged so that parents can attend the meetings in a relaxed manner knowing their children are safely cared for and entertained.

Given the high number of children and vulnerable adults expected to attend and the range of needs these individuals will have, the activity programme will be varied. We are hoping to allow some children and vulnerable adults to go out for periods of the day and others will have activities provided within the hotel complex.

Please note that it may not be possible for us to take out 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. Individuals will be assessed on an individual basis and any decision will be dependant on whether we are able to secure volunteers who have the experience to provide the level of care they require.

Evening Childrens & Vulnerable Adult care /Entertainment
Volunteers will be providing care on the Friday evening to allow parents to attend the gala dinner. Please note that 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.

Volunteers will be at the conference throughout the expert meeting to support the children's and vulnerable adults programme; each volunteer has completed an application, two references and received clearance from the Criminal Records Bureau. Each volunteer is allocated a child/children/vulnerable adult and given all the information needed about the individual's needs. All parents/carers will be consulted over their child/children's needs and a care plan drawn up where needed. Volunteers receive training on moving and handling, advice on child protection and the Society's policy on Volunteer Conduct and are informed about the importance of respect and encouraging independence.

Childcare volunteers will be allocated on a first come, first served basis. If your child has complex healthcare needs please contact us before booking to discuss needs and resources required.

The Venue

The expert meeting venue is the Hilton Hotel Northampton, which is an open plan, spacious hotel. The hotel is ideally located just off Junction 15 of the M1 in the English countryside of Northamptonshire. **The bedrooms are spread over two levels and the ground floor bedrooms will be allocated on a first come first serve basis, with priority for ground floor access given to the more severely disabled as there is no lift to the first floor, and those booking for the whole expert meeting.** The bedrooms at the hotel can take between 2 and 5 people per room.

The hotel benefits from its own 18-metre indoor, heated swimming pool and Livingwell Health Club consisting of Sauna, Steam Room and Spa to relax in or, for the energetic type, the hotel offers a fully equipped gymnasium which will be available to all guests over the course of the weekend. Guests arriving on the Friday will be able to use the hotel facilities, however bedrooms are not guaranteed to be ready until 3pm.

Hilton Hotel

100 Watering Lane
Collingtree
Northampton, NN4 0XW
Tel: 01604 700 666
www.hilton.co.uk/northampton



Friday 27 August 2010

10 am - 12 noon REGISTRATION

12 noon LUNCH

UNDERSTANDING ASPECTS OF SANFILIPPO DISEASE

1pm Chair: *Bryan Winchester and Judy Holroyd*

 Welcome and Setting the Scene: *Christine Lavery*

 Understanding the Biochemistry of MPS III: *Bryan Winchester*

 How is Sanfilippo Inherited? Carrier Testing and Reproductive Choices: *Sally Davies*

 Clinical Overview of Sanfilippo Types A, B, C and D: *Ed Wraith*

 Questions and Answers

LIVING WITH SANFILIPPO DISEASE

 Zara has MPS IIIA: *Anne Marie Watson*

 My Sister Roshani has MPS IIIB: *Keshini Nonis*

 An experience of MPS IIIC: *Evelyn Jarvie*

 Living with my Brother and Sister who have Sanfilippo Disease: *Hannah Donegani*

 We Adapted our Home for Oliver: *Karen Robinson*

3.10pm COFFEE BREAK

CLINICAL MANAGEMENT OF SANFILIPPO DISEASE

3.30pm Chair: *Uma Ramaswami and Chris Hendriksz*

 Managing Absences and Seizures: *Philip Jardine*

 Sleep Disturbance and Challenging Behaviour in Sanfilippo Disease: *Gill Moss*

 Brain and Behaviour in MPS III: *Elsa Shapiro*

 Indications and Benefits of having a Gastrostomy: *Rebecca Howarth*

 What to Expect from Adolescence and Beyond: Managing End Stage Disease in Sanfilippo: *Chris Hendriksz*

 Sanfilippo - a Triphasic Disease - Supporting the Family: *Debbie Box*

 Questions and Answers

5pm CLOSE

5.30pm Children and Vulnerable MPS III Adults Tea

6pm Childcare Volunteer Dinner

7 - 11pm Child and Vulnerable Adults Care Programme

7.30 - 11pm Gala Dinner

Saturday 28 August 2010

THE CHALLENGES OF TRANSLATING RESEARCH INTO TREATMENT

Chair - Fiona Stewart and Maurizio Scarpa

- 9am Waiting for Therapies from the MPS III families perspective - setting the scene:
Fiona Stewart
- The role of animal models in finding effective therapies for Sanfilippo disease:
Alex Langford-Smith
- How we get from the mouse to the Sanfilippo child from a regulatory perspective:
Aidan Gill
- The challenge of the blood brain barrier: *David Begley*
- Longevity and Mortality data for MPSIIIA, MPSIIIB, MPSIIIC and MPSIIID: *Simon Jones*
- Incidence and Natural History of MPS III in France and comparison with UK and Greece: *Marc Tardieu*
- Haemopoietic Stem Cell Transplant: the European and North American Experience:
Muhammad Saif
- Questions and Answers
- 11am COFFEE BREAK
- 11.15 FOUR ABSTRACTS SELECTED FOR ORAL PRESENTATION
- 12.15 LUNCH
- CURRENT RESEARCH PROGRAMMES FOR SANFILIPPO DISEASE
- 1.30pm Substrate reduction and optimisation studies: *Brett Crawford*
- New perspectives for crossing the Blood Brain Barrier in MPS III: *Maurizio Scarpa*
- Developing small molecules for the Sanfilippo diseases: *Brett Crawford*
- Gene modified Haemopoietic Stem Cell Transplant in the MPS mouse: *Brian Bigger*
- Gene Therapy for MPSIIIA - Where are we now?: *Olivier Danos*
- Gene Therapy for MPSIIIB - Where are we now?: *Jean Michel Heard*
- Genistein: A substrate reduction therapy for Sanfilippo disease: *Brian Bigger*
- Intrathecal Enzyme Replacement Therapy for MPSIIIA: *Charles Richards*
- Managing a Clinical Trial: *Simon Jones*
- DISCUSSION - Where do we go from here - Next step
- 4.30pm CLOSE

Deadline for bookings

Friday 23 July 2010

Please note that the above deadline will apply to all MPS Society members with the exception of new members and families that have just recently joined the Society's membership.

UK Resident MPS Society Family Registration

Surname	First Name	Special Dietary Requirements	Wheelchair (Tick)	DOB If under 17	MPS type
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/>	<input type="text"/>	<input type="text"/>
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/>	<input type="text"/>	<input type="text"/>
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/>	<input type="text"/>	<input type="text"/>
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/>	<input type="text"/>	<input type="text"/>
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/>	<input type="text"/>	<input type="text"/>
<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="checkbox"/>	<input type="text"/>	<input type="text"/>

ACCOMMODATION

The MPS Society has subsidised the prices below for MPS members based on a maximum of two adults. Additional adults are welcome at a charge of £60 when sharing a family room. Please see professionals and additional adults registration for additional room rates.

Family rooms accommodate a maximum of five persons per room and comprise of two double beds and one single foldaway bed. The family price is for two adults, MPS sufferers and siblings under 18 years of age. The price for an additional adult sharing this room is £60 per head.

Single room can only be offered at this price to those members attending the weekend alone and cannot be offered to additional adults who are accompanying other family members. Please see professionals and additional adults registration for additional room rates.

Double/twin room (a double room comprises one double bed and a twin room comprises one double bed and one foldaway bed. Maximum two persons, must include one adult).

If you are unsure or this doesn't fit your requirements, please contact MPS before completing your form.

As there are no lifts, to allow more immobile MPS III individuals to have priority to groundfloor rooms can your child or vulnerable adult be accommodated on the first floor of the hotel? Please circle yes or no.	YES / NO
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OVERNIGHT* ACCOMMODATION PACKAGE

Single £60 Double £120 Twin £120 Family £140 Additional adult £60 each

*Overnight accommodation comprises Friday lunch, Friday Gala Dinner and Friday night accommodation, Saturday breakfast and lunch and Friday and Saturday conferences

FRIDAY CONFERENCE ONLY

Adult £20

SATURDAY CONFERENCE ONLY

Adult £20

SATURDAY CONFERENCE

Inc. lunch £40
does not include accommodation

Please note that Friday and Saturday conference is for adults only. We are unable to accommodate children on the childcare programme when not booking the overnight accommodation package.

Additional information/comments to assist us with your booking.
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Overseas MPS Families Registration

Surname	First Name	Special Dietary Requirements	Wheelchair (Tick)	DOB If under 17	MPS type

ACCOMMODATION

The prices below are based on a maximum of two adults. Additional adults are welcome at a charge of £120 when sharing a family room. Please see professionals and additional adults registration form for additional room rates.

Single rooms can only be offered at this price to those attending the conference alone and cannot be offered to additional adults who are accompanying other family members. Please see professionals and additional adults registration form for additional room rates.

Family rooms accommodate a maximum of five persons per room and comprise of two double beds and one single foldaway bed. The family price is for two adults, MPS sufferers and siblings under 18 years of age. The price for an additional adult sharing this room is £120 per head.

Double/twin room (a double room comprises one double bed and a twin room comprises one double bed and one foldaway bed. Maximum two persons, must include one adult).

If you are unsure or this doesn't fit your requirements, please contact MPS before completing your form.

As there are no lifts, to allow more immobile MPS III individuals to have priority to groundfloor rooms can your child or vulnerable adult be accommodated on the first floor of the hotel? Please circle yes or no.

YES / NO

OVERNIGHT* ACCOMMODATION PACKAGE

Single £120 Double £240 Twin £240 Family £280 Additional adult £120 each

*Overnight accommodation comprises Friday lunch, Friday Gala Dinner and Friday night accommodation, Saturday breakfast and lunch and Friday and Saturday conferences

FRIDAY CONFERENCE ONLY

Adult £40

SATURDAY CONFERENCE ONLY

Adult £40

SATURDAY CONFERENCE

Inc. lunch £80
does not include accommodation

Please note that Friday and Saturday conference is for adults only. We are unable to accommodate children on the childcare programme when not booking the overnight accommodation package.

Additional information/comments to assist us with your booking.

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Methods of Payment

Please tick one of the boxes below to indicate which method of payment you wish to use.

- Pay the full amount by cheque or credit card when sending in the booking form.
- Send in the minimum deposit of £40 with the booking form, and then pay the rest of the amount owed in a lump sum no later than **Friday 2 July 2010**.
- Send in the minimum deposit of £40 with the booking form, and then pay the rest of the amount owed in equal monthly instalments and being paid in full by **Friday 2 July 2010**.
- Send in the minimum deposit of £40 with the booking form, and then apply for the Financial Assistance Scheme. In applying for Financial Assistance, the Society holds the right to apply to other charities for funding on your behalf, and only when this has been exhausted will the Society consider providing the funding. Should the full amount, including the deposit, be achieved from funding, the deposit that was made with the booking will be refunded. All bookings requiring financial assistance must be received by **Friday 2 April 2010**. Only available to UK-resident MPS Society families.

AMOUNT ENCLOSED (Minimum deposit £40): £

BALANCE TO PAY BEFORE FRIDAY 2 JULY 2010: £

Address Details

Name

Address

Post Code

Telephone Email

Payment Details

Payment by cheque Debit/Credit Card (Maestro, Solo, Delta, Visa, Mastercard) Security no. on reverse of card

Card Number Issue Number

Name on Card Valid From Expiry Date

Numbers in postcode (e.g. if your postcode is AB12 3BA, write 123)

Numbers in address (e.g. if you live at Flat 41, 315 Any Street, write 41315)

Professionals and Additional Adults Registration

Name/s	<input type="text"/>	
Organisation	<input type="text"/>	
Address	<input type="text"/>	
	<input type="text"/>	
Telephone	<input type="text"/>	Fax <input type="text"/>
Email	<input type="text"/>	

Overnight Accommodation Package

Overnight accommodation package comprises Friday lunch, Friday gala dinner, Friday night accommodation, Saturday breakfast and lunch and Friday and Saturday conference.

Price is per person

Single occupancy	£140	<input type="checkbox"/>
Two people sharing double room	£245	<input type="checkbox"/>
Two people sharing twin room	£245	<input type="checkbox"/>

Non-Residential Conference Options

Friday Conference Only (including lunch)	£40	<input type="checkbox"/>
Friday Conference Only (including lunch and Gala Dinner)	£65	<input type="checkbox"/>
Saturday Conference Only (including lunch)	£40	<input type="checkbox"/>

Special Dietary Requirements..

Any other requirements...

TOTAL AMOUNT DUE: £

Please Invoice Hospital or Institution (Please Tick)

Name	<input type="text"/>	Organisation	<input type="text"/>
Address	<input type="text"/>		
	<input type="text"/>		
Telephone	<input type="text"/>	Email	<input type="text"/>

Payment Details

Payment by cheque	<input type="checkbox"/>	Debit/Credit Card (Maestro, Solo, Delta, Visa, Mastercard)	<input type="checkbox"/>	Security no. on reverse of card	<input type="text"/>	<input type="text"/>	<input type="text"/>			
Card Number	<input type="text"/>			Issue Number	<input type="text"/>	<input type="text"/>				
Name on Card	<input type="text"/>	Valid From	<input type="text"/>	<input type="text"/>	<input type="text"/>	Expiry Date	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>
Address of Card holder	<input type="text"/>							Post Code	<input type="text"/>	