Recordati Rare Diseases Fondation d'entreprise

Inherited disorders of metabolism affecting the liver: management and outcome
Leuven (Belgium)
8 – 9 March 2018

COURSE DETAILS

Course description

A course jointly organised by the Hepatology and Metabolic Center, Leuven and the Charles Dent Metabolic Unit, London. This course will include up to date, state of the art lectures and debates, with expert European speakers, regarding current evidence for predicting / preventing complications, and treatment, including transplant and its outcomes, in inherited liver disease. Disorders covered will include the glycogen storage disorders, disorders of glycosylation, lysosomal acid lipase deficiency, disorders of bile acid synthesis, the organic acidemias, Wilson disease and mitochondrial disease. The latest advances in gene therapy and stem cell therapy for metabolic liver disease and the role of circulating microRNAs will also be discussed. Participants will be encouraged to bring challenging and/or informative cases for discussion.

Learning objectives

- To be aware of the complications associated with inherited disorders of the liver.
- To be aware of up to date evidence on current options for treatment, and their outcomes.
- To consider current and potential future methods of predicting or preventing complications.

Scientific Organising Committee

- Dr David Cassiman, Leuven
- Dr Elaine Murphy, London
- Dr John Walter, Manchester

Target audience and participant profile

The course is aimed at:
Consultants, senior trainees and scientists with a specific interest in inherited metabolic disease, hepatology or liver transplant. Paediatric or adult practice.

Fees

The course fees of **450€** cover:

- 2 nights hotel accommodation (7th and 8th March) including breakfast.
- Lunch, coffee and dinner during the course.

A local fee of **315€** is granted if accommodation is not needed.

Participants are responsible for their own travel arrangements to and from the course.
Fees are not refundable.

Registration process and deadline

The registration form should be completed on-line: **www.rrd-foundation.org** and submitted with your curriculum vitae in English.
No payment is required at this stage.
Deadline for registration is **10th of January 2018**.

Selection criteria and review process

Candidates will be selected based on their background, experience and geographical breakdown.
The scientific organising committee will review the applications and select participants.
Selection decisions will be announced within 10 days following the deadline for registration.

Accreditation

An application will be made for European CME (EACCME).

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PROGRAMME

Thursday 8 March:

Start of the meeting at 09:00

Session 1: Chair: David Cassiman

Single and multiple hepatic adenomata in GSD I – remove, or watch and wait?
Jessica Zucman-Rossi, Paris

Preventing cirrhosis in glycogen storage disorders – what is the evidence?
Aileen Marshall, London

Debate: More children with GSD should be offered a liver transplant.
For: Roshni Vara, London
Against: Helen Mundy, London

Session 2: Chair: Hannah Van Malenstein

Disorders of bile acid synthesis
Jörg Jahnel, Graz

Optimising liver transplant in disorders of intoxication: PA, UCD and MSUD
Roshni Vara, London

Gene therapy for metabolic liver disease
Julien Baruteau, London

Session 3: Chair: Helen Mundy

Long-term complications in GSD – pathogenesis, prevalence and prevention:

- Lipid burden alleviation prevents hepatic and renal complications in GSDIa in mice
  Fabienne Rajas, Lyon
- Reduced BMD
  Elaine Murphy, London
- Dyslipidemia
  Charlotte Lubout, Groningen

Role of circulating microRNAS in liver disease
Christoph Roderburg, Aachen

Participant cases with discussion (X2 cases).

Friday 9 March:

Start of the meeting at 09:00

Session 4: Chair: Elaine Murphy

0900
Participant cases with discussion (X3 cases).

The liver in multisystem disease: mitochondrial disorders
Shamima Rahman, London

The liver in multisystem disease: congenital disorders of glycosylation
Peter Witters, Leuven

Can we predict development of cirrhosis and acute hepatic failure in adults with LAL deficiency?
David Sheridan, Plymouth

Patient / support group perspective

Updates on Wilson disease and haemochromatosis
Bill Griffiths, Cambridge

Stem cell therapy in metabolic liver disease
Sabine Fuchs, Utrecht

End of the meeting around 16:00

Registration: ***WWW.RRD-FOUNDATION.ORG***

Contact: ***CKELLQUIST@RRD-FOUNDATION.ORG***