

## **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 (7<sup>th</sup> and 8<sup>th</sup> 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](http://www.rrd-foundation.org) and submitted with your curriculum vitae in English.

No payment is required at this stage.

Deadline for registration is **10<sup>th</sup> 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).

## 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 GSD1a in mice

*Fabienne Rajas, Lyon*

- Reduced BMD

*Elaine Murphy, London*

- Dyslipidemia

*Charlotte Lubout, Groningen*

Role of circulating microRNAs in liver disease

*Christoph Roderburg, Aachen*

Updates in LSD related liver disease: Niemann Pick B and Gaucher disease.

*Annalisa Sechi, Udine*

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](http://WWW.RRD-FOUNDATION.ORG)

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