ESPGHAN

European Society for
Paediatric Gastroenterology,
Hepatology and Nutrition

Targeting liver disease at DNA level - Venice 2015

Scientific program

Thursday, 29th Oct 2015

13.45

Welcome (Lorenzo d´Antiga, Federico Mingozzi, Ulrich Baumann)

Session 1: Next generation sequencing in liver disease

Chair: Ulrich Baumann

14.00-14.25

Overview: How novel diagnostic tools may change the management of monogenic liver disease. Patrick McKiernan, Birmingham

14.30-16.30

Centre specific presentations: clinical aspects (10-15 minutes each)

Utrecht: Roderick Howen

London: Roshni Vara

Bergamo: Maria Iascone

Hannover: Eva Pfister

Paris: Francois Petit

Newcastle: Robert Taylor

16.00-17.00

Panel discussion: Technical and clinical limitations of NGS

Moderators: Lorenzo D'Antiga, Ulrich Baumann, Thomas Illig, Maria Iascone

17.00-17.30 Coffee break

Session 2: Epigenetics

Chair: Lorenzo d´Antiga

17.30-18.15

Free papers

Gene therapy in tyrosinemia mouse model by targeted integration. Norman Junge

Usefullness of next generation sequencing analysis in clinically unsolved cases. Laura Pezzoli

Low GGT cholestasis with dysmorphic features and motor delay. Cristina Gonçalves

18.15-18.45

Lecture: Known genes with new diseases, and known diseases with new genes. Richard Thompson, London

Discussion

20.15 Social program and dinner

Friday, 30th October 2015

Session 3. Therapeutic approaches to paediatric liver diseases

Chair: Federico Mingozzi

8.00-8.20

Gene therapy: an overview. Fulvio Mavilio, Paris

8.20-8.50

Exploiting RNAi for treatment of genetic disorders, focus on A1ATD. Alfica Sehgal, Cambridge, Massachusetts

8.50-9.20

Antagomir based intervention in metabolic liver disease. Dirk Grimm, Heidelberg

9.20-9.45 Coffee break

9.45-10.15

Lentiviral gene transfer to the liver. Luigi Naldini, Milan

10.15-10.45

AAV8 based gene therapy for haemophilia B. Amit Nathwani, London

10.45-11.10

AAV8 based gene therapy for Crigler Najjar syndrome. Federico Mingozzi, Paris

11.10-11.40

Experience with acute intermittent porphyria. Gloria González, Pamplona

11.40-12.00

Scientist´s view: Aspects of feasibility, efficacy and safety of vector based gene therapy. Andres Muro, Trieste

12.00-13.00

Clinician's view: Challenges of gene therapy for monogenic diseases

Urea cycle defects. Ana Morais Lopez, Madrid

Organic acidemias. Carlo Dionisi Vici, Rome

Glycogen storage disorders. Philippe Labrune, Paris

Lysosomal storage disorders. Hidde Huidekoper, Rotterdam

13.00-13.30

Discussion and summary. Federico Mingozzi, Piter Bosma, Ulrich Baumann

13.30-14.00 Lunch

14.30-15.30

Invited attendees: guided discussion. Ulrich Baumann, Federico Mingozzi, Lorenzo D'Antiga

15.30-16.00 Coffee break

16.00-17.00

Summary and outlook

17.00 Farewell and departure