

4th Orphan Disease Symposium



Consensus Conference on Liver and Liver Cell Transplantation in Inborn Errors of Metabolism

26 - 27 January 2006

Brussels Belgium

Under the auspices of



SSIEM



Scientific Committee

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James Leonard (UK) – Mustapha Najimi (Belgium)
Hélène Ogier (France) - Marco Spada (Italy)



Cliniques Universitaires Saint-Luc
Université Catholique de Louvain



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www.pediatrie.be/orphan2006.htm

Foreword of the President

Dear Colleagues and Friends,

The results of liver transplantation have constantly improved and long-term survival now exceeds 90%. Better knowledge of immunosuppression has abolished most of its side-effects. Liver transplantation has therefore emerged as one of the most efficient treatments for many inborn errors of metabolism and the new frontiers will be discussed.

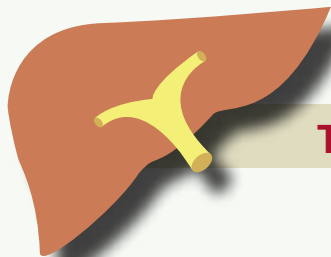
Cell therapy is in its early clinical application phase. Around fifty patients have received liver cell transplantation for various liver based conditions. Experimental studies in animal support the efforts to bring the procedure to the patient's bed. Inborn errors of the liver metabolism are high targets, and joined efforts between hepatologists and metabolicians will help to identify the best candidates to this innovative treatment.

Our meeting will gather all physicians involved in the care of these patients with the best researchers in the field.

We welcome you in Brussels !

Professor Etienne Sokal

President of the 4th Orphan Disease Symposium



Thursday 26 January 2006

13.30-14.30 Registration

14.30-14.40 Welcome address

CURRENT TREATMENT

Chair : Etienne SOKAL

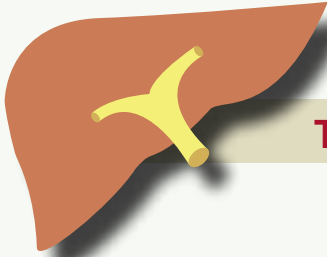
- 14.40-15.10 Medical progress and pitfalls of treatment of inborn errors of metabolism
James Leonard, Oxford, UK
- 15.10-15.30 Indications and results of liver transplantation for metabolic disorders
Deirdre Kelly, Birmingham, UK
- 15.30-15.50 Long-term consequences of immunosuppression
Raymond Reding, Brussels, Belgium
- 15.50-16.10 Medical versus transplant management of PFIC
Irena Jankowska, Warsaw, Poland
- 16.10-16.30 Metabolic causes of fulminant hepatic failure
Rob Squires, Pittsburg, USA

16.30 - 16.50 Coffee-Break & Posters viewing

PERSPECTIVES OF TREATMENT

Chair : Vincent BOURS

- 16.50-17.10 Results of liver cell therapy for metabolic disorders
Anil Dhawan, London, UK
- 17.10-17.30 Adult stem cells: perspectives in treatment of metabolic diseases
Maren Schulze, Kiel, Germany
- 17.30-17.50 In vitro tissue expansion perspectives
Mustapha Najimi, Brussels, Belgium



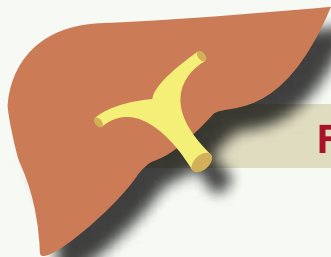
Thursday 26 January 2006

FREE COMMUNICATIONS

- 17.50-18.00 Immunological considerations in liver cell transplantation for Crigler-Najjar syndrome
Katrina Allen, Melbourne, Australia
- 18.00-18.10 Pre-clinical approach of hepatocyte transplantation in non-human primates: Efficient hepatocyte engraftment after partial portal embolisation
Anne Weber, Kremlin-Bicêtre, France
- 18.10-18.20 Effect of glyceril trinitrate on hepatocytes engraftment and metabolic correction in the MDR2-/- mouse model of progressive familial intrahepatic cholestasis
Lyes Boudechiche, Kremlin-Bicêtre, France
- 19.30 Official Dinner

Guided visit followed by a dinner at the Museum "Belgian Centre for Comic Strip Art"





Friday 27 January 2006

08.00 Opening Registration Desk

08.30-08.50 Progress and pitfalls in organic aciduria

Hélène Ogier, Paris, France

08.50-09.00 Report of liver transplantation in organic aciduria

Marco Spada, Turin, Italy

09.00-09.20 Long-term outcome in urea cycle disorders

Claude Bachmann, Lausanne, Switzerland

09.20-09.35 Results of liver cell transplantation in urea cycle disorders

Françoise Smets, Brussels, Belgium

09.35-09.45 Free communication: Neonatal hemochromatosis: 12-years experience of a liver transplant

Enke Grabhorn, Hamburg, Germany

09.45-10.15 ROUND TABLE

Marie-Cécile Nassogne & Anil Dhawan

10.15-10.45 Coffee-Break & Posters Viewing

FREE COMMUNICATIONS

10.45-10.55 Liver transplantation in metabolic diseases: the experience of a metabolic unit

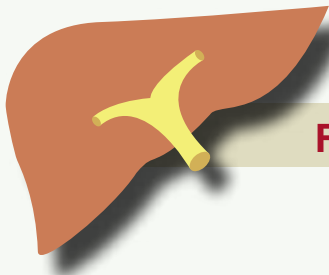
Rossella Parini, Milan, Italy

10.55-11.05 Sequelae of underlying disease and posttransplant complications in liver transplantation for metabolic disorders

Martin Burdelski, Hamburg, Germany

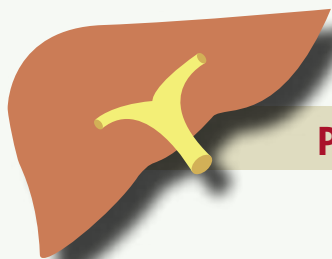
11.05-11.15 Experiences with NTBC and liver transplantation in tyrosinemia type I in the Netherlands

Françjan van Spronsen, Groningen, the Netherlands



Friday 27 January 2006

- 11.15-11.35 Liver and liver cell transplantation for glycogenosis
Maurizio Muruca, Rome, Italy
- 11.35-11.55 Preemptive liver transplantation for primary oxaluria
Pierre Cochat, Lyon, France
- 11.55-12.15 Neurological Wilson disease and liver transplantation
Jean-Charles Duclos-Vallée, Paris, France
- 12.15-12.45 ROUND TABLE**
Marco Spada & Jean de Ville de Goyet
- 12.45-13.45 Lunch & Posters viewing**
- 13.45-14.05 Crigler Najjar and liver transplantation
Maarten Sinaasappel, Rotterdam, The Netherlands
- 14.05-14.25 Liver or liver cell transplantation for phenylketonuria ?
Philippe Goyens, Brussels, Belgium
- 14.25-14.45 Indications of transplantation in respiratory chain disorders
Etienne Sokal, Brussels, Belgium
- 14.45-15.15 ROUND TABLE**
Deirdre Kelly & Jean-Bernard Otte
- 15.15 Closing Remarks**
- Farewell Drink & Award for the Best Communication



Poster Sessions

Lectin-reactive alpha-fetoprotein in the liver transplant assessment of patients with tyrosinaemia type I. Baumann U, Duhme V, Mc Kiernan PJ, Holme E. Birmingham Children's Hospital, Birmingham, UK

Hepatocellular tumors in metabolic disorders. Richter A, Grabhorn E, Ganschow R, Burdelski M. Pediatric Gastroenterology and Hepatology, University Medical Center Hamburg-Eppendorf, Germany

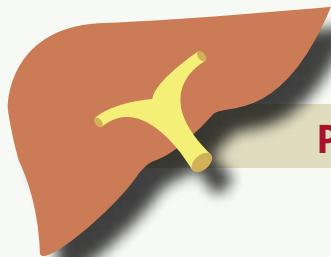
Does long-term phototherapy affect the liver in patients with Crigler-Najjar syndrome? Deutschmann A¹, Ganschow R², Schäfer H², Burdelski M²
¹Department of Paediatrics, Medical University of Graz, ²Department of Paediatrics, University Hospital Hamburg-Eppendorf, Germany

Long-term results of pre-emptive liver transplantation in primary hyperoxaluria type I. Beckmann M, Kemper MJ, Nolkemper D, Ganschow R, Burdelski M. Departments of Pediatrics and Hepatobiliary Surgery, University Medical Center Hamburg-Eppendorf, Germany

Mutation analysis of the G6PT gene in Russian patients with GSD1B: identification a novel mutation. Lomonosova E, Povalko N. Department of Inherited Metabolic Diseases, Research Centre for Medical Genetics, Russian Academy of Medical Sciences, Moscow, Russia

Hepatic transplantation in methylmalonic acidemia: a case report. Fuoti M, Pinotti M, Villa MC, Celano MR, Miceli V, Amoroso C, Rossi G, Parini R, Nebbia G. Clinica Pediatrica e Centro Trapianti Fegato, Fondazione Policlinico, Mangiagalli, Regina Elena, Milano, Italy.

Cryopreservation of murine hepatocytes induces mitochondrial membrane potential alteration without induction of caspase 3 activity. Stéphane X¹, Guigas B², Najimi M¹, Hue L², Sokal EM¹. Université Catholique de Louvain, ¹Laboratoire d'Hépatologie Pédiatrique et de Thérapie Cellulaire, ²Unité Hormones et Métabolisme, Brussels, Belgium



Poster Sessions

Preventive treatment of renal insufficiency with hyperhydration in 10 children with hyperoxaluria type I (HP I). Gagnadoux MF, Salomon R, Lacaille F, Boudaillez B, Taque S, Bertheleme JP, Guyot C, Niaudet, P. Hôpital Necker-Enfants malades, Paris, CHU d'Amiens, Centre Perharidy de Roscoff, CHU de Nantes, CHU de Rennes, France

Twelve years follow-up of liver transplantation (LT) for propionic acidemia. Lacaille F, Laurent J, de Lonlay P, Salomon R, Saudubray JM. Department of Paediatrics, Hôpital Necker-Enfants malades, Paris, France

A 2,3 kg liver recipient for neonatal hemochromatosis: a case report. Stroppa P, Candusso M, Bravi M, Melzi ML, Torre G, Colledan M. Pediatric liver transplantation Centre, Ospedali Riuniti-Bergamo-Italy.

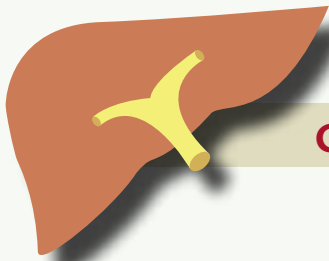
Liver transplantation in children with metabolic and genetic disorders: experience of one center. Stroppa P, Bosisio, Bravi M, Melzi ML, Candusso M, Torre G. Pediatric liver transplantation Center, Ospedali Riuniti-Bergamo-Italy

Endodermal progenitors from salivary gland for repopulation of liver and treatment of metabolic disorders. Endo F, Hisatomi Y, Okumura K, Matsumoto S, Satoh A, Hattori K, Nakamura K. Department of Pediatrics, Graduate School of Medical Science, Kumamoto University, Honjo 1-1-1, Kumamoto 860-8556, Japan

Is liver transplantation indicated in metabolic disorders with neurological involvement? Two case reports. Verloo P, De Meirleir L, Van Coster R, Seneca S, Smet J, Van Biervliet S, Van Winckel, M. University Hospital Ghent, Belgium

Primary hyperoxaluria type one - Can foetal liver biopsy and later hepatorenal transplant still be the treatment options? Patwardhan A, James Cook University Hospital Middlesbrough, Higgins C. Royal Wolverhampton. Place were work Done- Royal Wolverhampton

Liver transplant in inherited metabolic disorders in Portugal. Veiga C, Garcia P, Diogo L, Gonçalves I. Liver Transplant & Metabolic Units, Paediatric Hospital & University Hospital, Coimbra, Portugal



General Information

Venue

The congress will be held at the Palais des Académies, which is very close to the Royal Palace and in the center of Brussels (Rue Ducale 1- 1000 Brussels)

Registration Fee

The fee includes participation in sessions, congress bag, program, abstract book, coffee-breaks, lunch and farewell drink on Friday

- Participant: 150€
- Young Investigator/Trainee: 75€
- Accompanying person: 50€

All payment must be made in Euro, by Bank Card (VISA, Amex, Mastercard) or bank transfer to the account 210-0669243-92 with the mention "447944" of Fortis, Rue Montagne du Parc, B-1200 Brussels (IBAN BE12 2100 6692 4392 – Swift (BIC) GEBABEBB)

Official Dinner

The Official Dinner will take place at 7.30 p.m at the Museum "Belgian Centre for Comic Strip Art" (Rue des Sables 20 - 1000 Brussels)

(Reservation required – 50€/person)

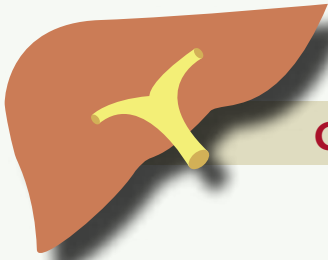
Meeting Place: 7.15 p.m. main hall of Palais des Académies

To reach the congress place

- National airport: Zaventem Brussels airport can be reached from most main cities around the world with direct daily flights
Shuttle to "Gare Centrale" (15 minutes walking to venue)
- Train station "Gare du Midi": Eurostar and Thalys
- Public transportation and taxis are easily available at the station and the airport (20 minutes from the venue)
- Subway station nearby venue: "Trône" - www.stib.be

Accreditation

A Belgian accreditation has been obtained: Ethics (6)- 90 UFC - 46000127



General Information

Museum



Congress Venue

1. Hotel President Centre, Rue Royale 160
2. Hotel Grand Place Arenberg, Rue d'Assaut 15
3. Hotel du Congrès, Rue du Congrès 42-44
4. Hotel Hesperia Grand Place, Rue des Colonies 10-12
5. Hotel Carrefour de l'Europe, Rue du Marché aux Herbes 110
6. Stanhope Hotel, Rue du Commerce 9

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