

Agenda for the meeting

FRIDAY 18 November 2011

09.00-09.30 Registration: *tea, coffee and snacks to be served upstairs*

09.30-09.45 Welcome to the meeting: new discoveries in alkaptonuria research
Dr Nicolas Sireau, AKU Society, UK

MORNING SESSION – chaired by Prof Jim Gallagher, University of Liverpool

09.45-10.15 From weed killer to wonder drug: the development of nitisinone
Prof Ted Lock, Liverpool John Moores University, UK

10.15-10.45 Biomarkers in alkaptonuria
Dr Anne-Christine Bay Jensen, Nordic Bioscience, Denmark

10.45-11.15 How collagen sticks together: NMR studies of collagen structure and what disrupts it
Wing Ying Chow, University of Cambridge, UK

11.15-11.30 BREAK *tea, coffee and snacks to be served upstairs*

11.30-12.00 Structure based drug discovery: enzyme repair therapy in alkaptonuria
Dr Neil Kershaw, University of Liverpool, UK

12.00-12.30 Protein replacement therapy
Dr Farid Kahn, Protein Technologies Ltd, UK

12.30-13.00 A non-viral S-MAR plasmid vector gene therapy for alkaptonuria
Dr Suet-Ping Wong, Imperial College London, UK

13.00-14.00 LUNCH *buffet lunch to be served upstairs*



5th INTERNATIONAL WORKSHOP ON AKU

FRIDAY 18 November 2011

AFTERNOON SESSION – chaired by Dr Jonathan Jarvis, University of Liverpool

- 14.00-14.30 Metabolomics in alkaptonuria
Dr Gordon Ross, Agilent, UK
- 14.30-14.50 Alkaptonuria in the mouse: blood biochemistry
Dr Andrew Preston, University of Liverpool, UK
- 14.50-15.15 Alkaptonuria in the mouse: ochronosis
Mr Craig Keenan, University of Liverpool, UK
- 15.15-15.30 BREAK *tea, coffee and snacks to be served upstairs*
- 15.30-15.55 Clinical and radiographic pictures of ochronotic arthropathy
Prof Jozef Rovenský, National Institute of Rheumatic Disease, Piešťany
- 15.55-16.20 A new potential target for nitisinone treatment in a mouse model:
oculocutaneous albinism type 1B
Prof Robert Aquaron, University of Marseilles, France
- 16.20-16.45 Understanding joint destruction in alkaptonuria
Dr Adam Taylor, University of Liverpool / Lancaster University, UK
- 16.45-17.10 AKU Severity Score Index
Dr Trevor Cox, Royal Liverpool University Hospital, UK
- 17.10-17.50 Plenary Discussion: 'Developments in alkaptonuria research'
Open discussion of the day's talks with questions from the floor, chaired by:
Prof Jim Gallagher, University of Liverpool
Dr Jonathan Jarvis, University of Liverpool
Dr L Ranganath, Royal Liverpool University Hospital
- 17.50-18.00 Close of the first day / Instructions for conference dinner
- 19.00-22.00 CONFERENCE DINNER: *Dining Room, Liverpool Medical Institution*

5th INTERNATIONAL WORKSHOP ON AKU

SATURDAY, 19 November 2011

- 09.00-09.30 Registration: *tea, coffee and snacks to be served upstairs*
- 09.30-09.40 Welcome to the second day: the importance of clinical studies and patient support
Dr Nicolas Sireau, AKU Society, UK

MORNING SESSION – chaired by Dr L Ranganath, Royal Liverpool University Hospital

- 09.40-10.00 Chemical measurements of alkaptonuria
Dr Sarah Hatch, Royal Liverpool University Hospital, UK
- 10.00-10.25 Questionnaire analysis of the alkaptonuria clinical evaluation study
Kate Williams, Royal Liverpool University Hospital, UK
- 10.25-10.45 Measurements of metals in alkaptonuria joint replacement surgery
Dr Ashley Newton, Royal Liverpool University Hospital, UK
- 10.45-11.15 Hot spots: alkaptonuria in Jordan
Dr Mohammed Alsbou, University of Mutah, Jordan
- 11.15-11.30 BREAK tea, coffee and snacks to be served upstairs
- 11.30-11.50 A growing problem: alkaptonuria in India
Dr Isaac Jebaraj, Christian Medical College, Vellore, India
- 11.50-12.15 Improving clinical assessment in alkaptonuria
Dr Wendy Introne, National Institutes of Health, USA
- 12.15-12.35 The use of physiotherapy in managing alkaptonuria
Dr Sophie Taylor, Royal Liverpool University Hospital, UK
- 12.35-13.00 Plans for a nitisinone trial in alkaptonuria
Dr L Ranganath, Royal Liverpool University Hospital, UK
- 13.00-14.00 LUNCH *buffet lunch to be served upstairs*



5th INTERNATIONAL WORKSHOP ON AKU

SATURDAY, 19 November 2011

AFTERNOON SESSION – chaired by Dr Nicolas Sireau, AKU Society

- 14.00-14.30 AKU video: 'Move to Improve'
Leona Wagner, AKU Society representative for Germany
- 14.30-15.00 The role of online communities in patient support
Robert Pleticha, EURORDIS, France
- 15.00-15.30 Working collaboratively for a national plan for rare diseases
Stephen Nutt, Rare Disease UK
- 15.30-15.45 BREAK *tea, coffee and snacks to be served upstairs*
- 15.45-16.45 PANEL DISCUSSION: Q&A WITH THE AKU RESEARCH TEAMS
Open questions from the floor to all the speakers. The panel will be headed by:
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| <u>University of Liverpool</u> | <u>Royal Liverpool University Hospital</u> |
| Prof Jim Gallagher – Lead on basic science | Dr L Ranganath – Lead on clinical science, UK |
| Dr Jonathan Jarvis – Lead on animal model | Mr John Davidson – Orthopaedic Surgeon |
| <u>National Institutes of Health</u> | <u>AKU Society</u> |
| Dr Wendy Introne – Lead on clinical science, USA | Dr Nicolas Sireau – Lead on patient support |
- 16.45-16.50 Announcement of 6th International Workshop on AKU, to be hosted by
Prof Rovenský at the Slovak Health Spa Piešťany, in the Slovak Republic
- 16.50-17.00 Close of Meeting / Thanks to the speakers

Presentations will be made available on the AKU Society website shortly after the meeting.

The presentations from the 4th Workshop are already available to download,
and summaries of the talks will be published in a special edition of the JIMD:
“Proceedings of the 4th International Workshop on AKU” as their December issue.