



Shining a light on Alport Syndrome

**3-5 January 2014
Oxford,
United Kingdom**

An exciting international event designed by Alport Syndrome individuals and families in partnership with the leading International experts to collaborate on ideas for the future.

There will be separate breakout sessions for national patient organisations.

Travel and accommodation bursaries available for a limited number of students in return for bringing a poster with some new ideas and input for our discussions.

A collaboration of:



The Organising Committee and Alport patient groups from across the world

Invite interested medical professionals, scientists, geneticists, academics, researchers:

The 2014 International workshop on Alport Syndrome

- Understand the latest findings and new ideas – hearing, kidneys (basic science and treatments), eyes, pharma solutions, clinical trials through access to alliance of national patient registries
- Collaborate with International colleagues in workshops on specific topics, e.g. novel approaches to treatment
- Present your own research or case studies
- Hear patient perspectives – a summary of a recent study
- Network with experts in related fields or related disease areas
- Be part of the future of Alport Syndrome research and treatment

What to do next...

Application process now open. Simply email your details (name, address, phone number and a short paragraph indicating your specific area of interest) to: research@alport.info by **7 September 2013**.

In your short paragraph, please indicate your particular activities which justify favourable consideration of you as a participant and contributor to this workshop. This information is important, as it allows the Workshop Organising Committee to make an informed decision when reviewing and accepting applications.

By applying you will receive details of how to register plus updates and information about the agenda.



Outline agenda and formats

Participants invited to arrive for lunch on Friday 3 January

To encourage open communication, each member of the workshop will need to agree that any information presented at this International workshop on Alport Syndrome, whether in a formal talk, poster session, or discussion, is a private communication from the individual making the contribution and is presented with the restriction that such information is not for public use. Prior to quoting or publishing any such information presented at the workshop in any publication, written or electronic, we will obtain written approval of the contributing member.

Friday afternoon: Current work and new ideas

- Information updates (in plenary) including summary of survey into patient views of Alport research: to share needs and what is currently being worked on
- Poster session/market place of ideas, followed by networking over dinner

Saturday – detailed working on 2 topics:

1 Basic science questions

- Basement membranes**
Formation and development of the glomerular basement membrane and cochlear basement membrane; differences in Alport Syndrome
- What do we know about treatment? (from animals and man)**
Animal models and what we've learned
Current therapies in patients
How disease-slowing therapies work
- What are the most promising new approaches?**
In animals: what is worth testing?
In man: candidate supplementary approaches
- What causes deafness in Alport patients and how might we prevent it?**
- What's in the eyes in Alport Syndrome?**
What might occur? How to see it?
What is the diagnostic value of looking?

2 Diagnosis and Clinical trials

- Diagnosis in the real world – what are the best ways to do this in 2014?**
Who benefits most from a genetic test?
What does the mutation tell us (genotype-phenotype correlation)
Current role of non-genetic testing (renal biopsy including fluorescence studies: skin, eye testing)
- How to recruit patients to trials**
Role of registries to underpin research
Alliance of patient groups and agreed ways of working together to support trials

Sunday: how do we bring it all together?

- From the ideas generated yesterday: What actions going forward?
What requires funding?
- Prepare a plan to present back to the national patient groups



Key contributors and Outputs from the workshop

Workshop Organising Committee

and key contributors confirmed

Professor Colin Baigent, MRC Scientist, Hon Consultant in Public Health, Oxford, UK

Professor Jie Ding, Department of Pediatrics, Peking University First Hospital, Beijing, China

Professor Frances Flinter, Consultant in Clinical Genetics, Guy's Hospital, London, UK

Dr Daniel Gale, UCL Centre for Nephrology, London, UK

Professor Oliver Gross, Department of Nephrology and Rheumatology, Georg-August-Universität Göttingen, Germany

Professor Clifford Kashtan, Professor, Department of Pediatrics, University of Minnesota, USA

Associate Professor Julian Midgley, Department of Paediatrics, Alberta Children's Hospital, Canada

Professor Jeffrey Miner, Washington University in St. Louis, USA

Mr Moin Mohamed, Consultant Ophthalmic Surgeon, St Thomas' Hospital, London, UK

Professor Judith Savige, University of Melbourne, Australia

Dr Judy Taylor, Consultant Paediatric Nephrologist, Evelina Children's Hospital, London, UK

Professor Neil Turner, Professor of Nephrology, University of Edinburgh and Consultant Nephrologist, Royal Infirmary of Edinburgh, UK

Representatives from the following national Alport family support organisations:

Dave Blatt, Alport Foundation of Australia

Susie Gear and Jules Skelding, Alport family support group, UK

Sharon Lagas, Alport Syndrome Foundation, USA

Daniel Renault, AIRG France/Europe

Outputs

Note: to be registered with Royal College of Physicians for Continuing Professional Development credits (number of hours to be confirmed)

- A fully documented written summary with photographs of key materials generated
- A new and comprehensive international Research Strategy for Alport Syndrome that covers all aspects eg kidneys, hearing, eyes, genetics
- A research plan, including:
 - Clear list of research priorities and need for funding
 - How to engage the future generations of scientists and researchers
- An alliance of national patient organisations and agreed ways of working together to support clinical trials and how to fund international research