



Agenda

3rd of November 2014

08:45 – 09:15 Gather together and On-site Registration

09:15 – 09:30 Introduction

David Hartley, The XLP Research Trust and Professor Bobby Gaspar, UCL Institute of Child Health, UK
Sumathi Iyengar- Amir Kedar, WAS organizations and Professor Adrian Thrasher, UCL Institute of Child Health, UK

09:30 – 10:00 - Plenary Session A (XLP and WAS)

Prof. Luigi Notarangelo – Harvard Medical School, Boston, USA.

"The Immune Dysregulation of WAS: Novel Insights"

10:00 – 10:30 - Plenary Session B (XLP and WAS)

Prof. Stuart Tangye – Garvan Institute of Medical Research, NSW, Australia.

"XLP: 1974 to the present day"

15 minute talks with 5 minutes discussion after each talk

10:40 – 11:40 -First WAS Session

Chair: Adrian Thrasher

10:40–11:00 Prof. Hans Ochs – Seattle Children's, USA.

"Reversion of WAS gene mutation: Does it affect immune function, and clinical phenotype?"

11:00–11:20 Dr. Daniele Moratto - University of Brescia, Italy

"Low platelet counts in female carriers of WAS mutations showing high level of X-inactivation of the non-mutated chromosome in hematopoietic precursors and myeloid-derived leukocytes"

11:20–11:40 Dr. Pamela Lee - Queen Mary Hospital, Hong Kong.

"Improving awareness and genetic diagnosis of Wiskott-Aldrich syndrome in Asia: A multi-centered collaborative study on 90 patients from the Asian Primary Immunodeficiency (APID) Network"

11:40 – 12:10 - Break

12:10 – 13:30 - Second WAS Session

Chair: Siobhan Burns

12:10–12:30 Dr. Pamela Lee - Queen Mary Hospital, Hong Kong.

"Wiskott-Aldrich Syndrome Protein Targets Enteropathogens to Autophagy and Limits Inflammasome Activation"

12:30 – 12:50 Dr. Mira Barda Saad – Bar Ilan University, Israel.

"The negative regulation of the Wiskott-Aldrich Syndrome protein"

12:50–13:10 Dr. Silvia Giliani -University of Brescia, Italy.

"Influence of WAS gene alterations on late stages of human B cell differentiation"

13:10–13:30 Dr. Brian Davis – University of Texas, USA.

"Genetic correction of induced pluripotent stem cells from a Wiskott-Aldrich Syndrome patient normalizes the immune defects"

10:40 – 11:40 - First XLP Session

Chair: Bobby Gaspar

10:40–11:00 Dr. Hirokazu Kanegane - University of Toyama, Japan.

"Whole exome sequencing reveals atypical phenotype of X-linked lymphoproliferative syndrome"

11:00–11:20 Prof. Stuart Tangye - Garvan Institute of Medical Research, NSW, Australia.

"Primary immunodeficiencies as models to delineate requirements for anti-EBV immunity"

11:20–11:40 Prof. Colin Duckett - University of Michigan Medical School, USA.

"Why do defects in XIAP, a protein with many cellular roles, lead to XLP2, and how can this guide our treatment of the disease"

12:10 – 13:30 - Second XLP Session

Chair: Claire Booth

12:10–12:30 Dr. Andrew Snow - Uniformed Services University, Bethesda, MD, USA.

"SAP facilitates recruitment and activation of LCK at NTB-A receptors during restimulation-induced cell death"

12:30–12:50 Dr. Pamela Schwarzberg - National Human Genome Research Institute, NIH, USA

"Defective Lymphocyte Interactions in XLP and other Primary Immunodeficiencies"

12:50–13:10 Dr. Philipp Jost - Technical University of Munich, Germany.

"XIAP restricts TNF- and RIP3-dependent cell death and inflammasome activation"

13:10–13:30 Prof. Andrea Graziani - Università Vita e Salute San Raffaele, Italy.

"Inhibition of Diacylglycerol kinase alpha rescues TCR-induced diacylglycerol signaling and restimulation induced cell death in XLP lymphocytes"

13:30 – 14:30 - Lunch

14:30 – 15:50 - Third WAS Session

Chair: Austen Worth

14:30 – 14:50 Dr. Steve Thomas -University of Birmingham, UK.

"Platelet actin nodules are podosome-like structures dependent on Wiskott-Aldrich Syndrome protein and Arp2/3 complex"

14:50 – 15:10 Dr. Lucia Sereni - San Raffaele Scientific Institute, Milan, Italy.

"Evaluation of platelet defect in a new mouse model lacking WASp in the megakaryocytic lineage"

15:10 – 15:30 Prof. Fabio Candotti - Division of Immunology and Allergy, CHUV, Switzerland.

"New Findings On Platelets And Red Blood Cells (RBC) In Wiskott-Aldrich Syndrome"

15:30 – 15:50 Prof. Hans Ochs – Seattle Children's, USA.

"Hematopoietic Stem Cell Transplantation for X-linked thrombocytopenia with mutations in the WAS gene"

14:30 – 15:50 - Third XLP Session

Chair: Kim Nichols

14:30 – 14:50 Dr. Sujal Ghosh - University Hospital Düsseldorf, Germany.

"ITK deficiency - clinical and immunological findings in nine patients"

14:50 – 15:10 Dr. Kim Nichols - St. Jude Children's Research Hospital, Memphis, USA.

"Janus kinase inhibition as a novel treatment for hemophagocytic Lymphohistiocytosis"

15:10 – 15:30 Prof. Rebecca Marsh - Cincinnati Children's Hospital, USA.

"Reduced Intensity Conditioning Regimens Containing Alemtuzumab, Fludarabine, and Melphalan Are Safe for Patients with XIAP Deficiency"

15:30 – 15:50 Dr. Troy Messick – The Wistar Institute, USA.

"Development of small molecule inhibitors of EBV for the treatment of XLP-associated diseases"

15:50 – 16:15 – Break

16:15 – 16:45 - Fourth WAS Session

Chairs: Sumathi Iyengar, Amir Kedar

The WAS Patient perspective

16:15 – 16:30 Mrs. Helen Mahony

16:30 – 16:45 Mrs. Rebecca Rolfe

16.15 – 16.45 – Fourth XLP Session

Chair: David Hartley

The XLP Research Trust – moving forward supporting the XLP research community.

Open discussion of future plans and direction for the charity.

16:45 – 17:30 - Plenary Session C (XLP and WAS)

Chair Adrian Thrasher

Prof. Mike Dustin – Kennedy Institute of Rheumatology, University of Oxford, UK.

"The Immunological Synapse"

17:30 – 18:30 – Welcome reception

An informal get-together to renew acquaintances and meet new colleagues will be held at the Meeting Venue. All registered participants are invited to join.



4th of November 2014 (Joined clinical session WAS and XLP)

08:30- 09:00 – Second day registration

10 minute talks with 5 minute discussion after each talk

09:00- 10:00- HSCT session

Chair: Bobby Gaspar

- Advances in HSCT – newer chemotherapeutic agents, prevention and treatment of GvHD, haplo-identical transplant, reduced intensity conditioning, mixed chimerism
- Long term outcome of HSCT

09:00 – 09:15 Dr. Paul Veys - Great Ormond Street Hospital for Children, London, UK

"Recent advances in stem cell transplantation for immunodeficiency diseases"

09:15 – 09:30 Dr. Philipp Agyeman - Great North Children's Hospital, Newcastle upon Tyne, UK.

"Successful outcome of HSCT for children with WAS using treosulfan and fludarabine – UK experience"

09:30 – 09:45 Dr. Michael Albert - Dr. von Hauner Childrens Hospital, Munich, Germany.

"Results of allogeneic stem cell transplantation from matched donors after myeloablative busulfan-based conditioning"

09:45 – 10:00 Panel discussion on the WAS HSCT treatment strategies

10:00 – 11:00 - Clinical manifestation and outcome

Chair: Hans Ochs

- Long term outcome for patients, QOL
- New treatments

10:00 – 10:15 Dr. Kim Nichols - St. Jude Children's Research Hospital, Memphis, USA.

"Treatment of Epstein-Barr Virus-induced hemophagocytic lymphohistiocytosis with rituximab containing chemo-immunotherapeutic regimens"

10:15 – 10:30 Prof. Anna Shcherbina - Federal Research and Clinical Center, Moscow, Russia.

"Treatment of thrombocytopenia in Wiskott-Aldrich Syndrome"

10:30 – 10:45 Dr. Nizar Mahlaoui - Centre de Référence National Déficits Immunitaires Héritaires (CEREDIH), Hôpital Necker-Enfants, Paris, France.

"Severe refractory thrombocytopenia Phenotype in patients with WAS and Role of a thrombopoietin receptor agonist"

10:45 – 11:00 Dr. Michael Albert - - Dr. von Hauner Childrens Hospital, Munich, Germany.

"Effect of splenectomy, stem cell transplantation, gene therapy or no definite treatment on frequency of disease related complications and physician-perceived quality of life in WAS"

11:00– 11:30 - Break



11:30 – 13:30 - Gene Therapy

Chair: Adrian Thrasher

- Advances in GT – Site specific gene corrections, Zinc finger nucleases, non-viral techniques
- Outcome of GT

11:30 – 11:45 Dr. Maximilian Witzel - The Gene Center of the University of Munich, Germany.
"Haematopoietic Stem Cell Gene Therapy for Wiskott-Aldrich Syndrome- The German trial"

11:45 - 12:00 Dr. Alessandro Aiuti - San Raffaele-Telethon Institute for Gene Therapy, Milan, Italy.
"Lentiviral gene therapy for WAS"

12:00 - 12:15 Dr. Anne Galy - Inserm Unit 951, Genethon, Evry, France.
Clinical Efficacy of Gene Therapy for Severe Wiskott-Aldrich Syndrome: Results of an international study.

12:15 - 12:30 Dr. Sung Yun Pai - Harvard Medical school, Boston, USA.
"Gene therapy for WAS: Results from Boston"

12:30 – 12:45 Dr. Claire Booth - Great Ormond Street Hospital for Children, London, UK.
"Gene therapy for XLP"

12:45 – 13:00 Dr. Toni Cathomen - Medical Center - University of Freiburg, Germany.
"Genome editing in iPSC -- from disease modeling to therapy"

13:00– 13:30 Open Discussion and Summary

Prof. Bobby Gaspar, Prof. Adrian Thrasher, UCL Institute of Child Health, London, UK

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