A Symposium for Researchers and Clinicians on

www.xlp-was2016.org

26th September, 2016 - London

Agenda

08:30 – 09:00 - Gather together and On-site Registration

09:00 – 09:15 - 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

09:15 – 09:45 - Plenary Session A (XLP and WAS)

Prof. Adrian Thrasher – ICH London, UK.

Wiskott Aldrich Syndrome: Advances in understanding of pathophysiology and development of novel therapies

09:45 – 10:15 - Plenary Session B (XLP and WAS)

Prof. Bobby Gaspar - ICH London, UK.

Gene therapy for PID - Lessons from ADA and Perforin deficiency

15 minute talks with 5 minutes discussion after each talk

10:25 – 11:25 -First WAS Session
Chair: Anne Galy

10:25-10:45 Dr. Fabio Candotti, CHUV, Lausanne, Switzerland

Mouse models of autoimmunity in WAS

10:45-11:05 Dr. Rongxin Dai, Children's Hospital of Chongqing Medical University, Chongqing, China

Abnormalities of follicular helper T-cell number and function in Wiskott-Aldrich Syndrome

11:05-11:25 Dr. Lisa Westerberg, Karolinska Institutet, Sweden.

Nuclear WASp regulates transcription networks in developing T lymphocyte

10:25 – 11:25 - First XLP Session
Chair: Claire Booth

10:25-10:45 Dr. Sylvain Latour, Institute Imagine , Paris, France

Inherited CTPS1 and CD70 deficiencies predispose to Epstein Barr virus infection.

10:45-11:05 Dr. Hirokazu Kanegane, TMDU, Tokyo, Japan

Selective dysregulation of Epstein Barr virus infection in hypomorphic ZAP70 mutation

11:05-11:25 Dr. Stuart Tangye , Garvan Institute of Medical Research, UNSW Australia

Combined immunodeficiency and Epstein-Barr virus-induced B cell malignancy in humans with inherited CD70 deficiency

11:25 – 11:45 - Break

11:45 – 12:45 - Second WAS Session
Chair: Fabio Candotti

11:45-12:05 Dr. Marton Keszei, Karolinska Institute, Sweden.

WASP is a key regulator of peripheral neutrophil functions

12:05-12:25 Dr. Brian R. Davis, Center for Stem Cell and Regenerative Medicine, UTHealth, Houston, Texas, USA

Somatic reversion in the Wiskott-Aldrich Syndrome

12:25-12:45 Dr. Daniele Moratto, Institute for Molecular Medicine, Brescia, Italy

Effect of atypical lyonization in females carrier of mutations in the WASP gene

11:45 – 12:45 - Second XLP Session
Chair: Sylvain Latour

11:45–12:05 Dr. Andrea Graziani University Vita e Salute San Raffaele, Italy.

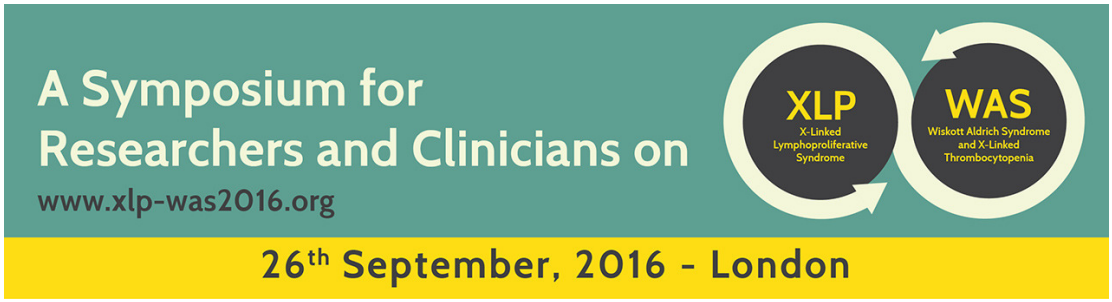
Inhibition of diacylglycerol kinase alpha restores estimulation-induced cell death and reduces immunopathology in XLP1

12:05–12:25 Dr. Gianluca Baldanzi, University of Piemonte Orientale, Italy.

Repurposing of existing drugs for XLP1 therapy.

12:25–12:45 Dr. Troy Messick The Wistar Institute, USA.

Development of a drug for the treatment of latent EBV.



12:45 – 13:45 - Lunch

13:45 – 14:45 - Third WAS Session
Chair: Lisa Westerberg

13:45-14:05 Dr. Mira Barda Saad, Bar Ilan University, Israel

Potential therapeutic approach for Wiskott-Aldrich Syndrome and X-linked Thrombocytopenia

14:05-14:25 Dr. Hanna Brauner, Karolinska Institute, Sweden.

Interleukin-2 is sufficient to restore killing capacity of Wiskott-Aldrich syndrome protein-deficient NK cells in vivo

14:25 – 14:45 Dr. Lucia Sereni, Italy

Intrinsic defect in WAS-/- platelets: studies in conditional mouse model and WAS gene therapy treated patients

14:45 – 15:30 - Fourth WAS Session - QOL
Chairs: Sumathi Iyengar, Amir Kedar

14:45 – 15:00 Dr. Michael Albert, Dr. von Hauner Childrens Hospital, Munich, Germany

Effect of treatment decisions on clinical outcome and quality of life in WAS

15:00 – 15:15 Dr. Robert Sokolic, Center for Biologics Evaluation and Research, FDA, USA

Quality of Life in patients with Wiskott-Aldrich Syndrome and X-linked Thrombocytopenia

15:15– 15:30 Family Cases Presentation

Mr. Wolfgang Luxa – Germany

Mrs. Kerry Tuffin - UK

13:45 – 14:45 - Third XLP Session
Chair: Stuart Tangye

13:45 – 14:05 Dr. Neelam Panchal, UCL Institute of Child Health, London, UK

T cell gene therapy for XLP

14:05 – 14:25 Dr. Benjamin Houghton, UCL Institute of Child Health, London, UK.

Targeted gene addition strategies for the treatment of X-linked lymphoproliferative disease

14:25 – 14:45 Dr. Pamela Schwartzberg, National Human Genome Research Institute, USA

Evaluation of Primary Immunodeficiencies Associated with an Inability to Clear Epstein-Barr Virus

14:45 – 15:30 - Fourth XLP Session
Chair: David Hartley

14:45 – 15:30 David Hartley, The XLP Research Trust

Setting up and XLP Patient Registry – status and support.

15:30 – 15:45 – Short Break

15:45 – 16:40 – Clinical Session I - HSCT (XLP and WAS)
Chair: Bobby Gaspar

15:45– 16:00 Dr. Franco Locatelli, Ospedale Pediatrico Bambino Gesu, Rome, Italy.

Clinical outcome and immune recovery after adoptive infusion of BPX501cells (donor T cells transduced with iC9 suicide gene) in children with Wiskott Aldrich Syndrome (WAS) given α/β T-cell depleted HLA haploidentical HSCT

16:00 – 16:10 Dr. Hans Ochs, Seattle Children's Hospital, Seattle USA.

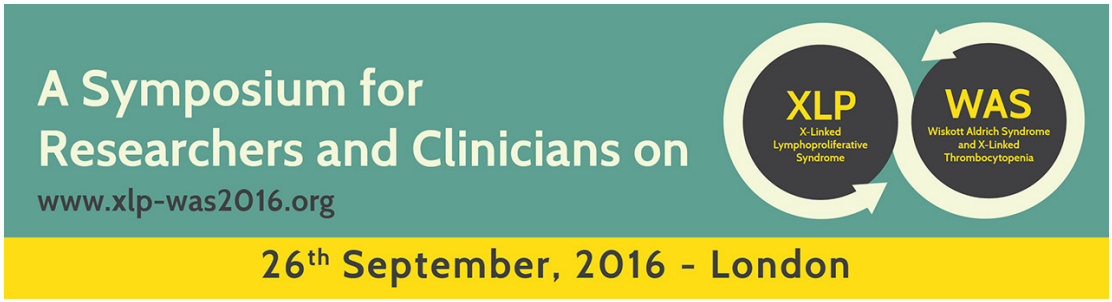
Knowah Case Study and discussion

16:10 – 16:25 Dr. Shintaro Ono, TMDU, Tokyo, Japan

Hematopoietic stem cell transplantation for XIAP deficiency in Japan

16:25 – 16:40 Dr. Kohsuke Imai, TMDU, Tokyo, Japan.

Two patients with Wiskott-Aldrich syndrome suffered from severe lung disease long after hematopoietic stem cell transplantation



16:40 – 17:25 – Clinical Session II – GT and other therapies (XLP and WAS)
Chair: Adrian Thrasher

16:40 – 16:55 Prof. Marina Cavazzana, Imagine Institute, Paris, France.
Gene therapy results of WAS : There is room for further improvements

16:55 – 17:10 Dr. Alessandro Aiuti, San Raffaele Telethon Institute for Gene Therapy, Milano, Italy.
TIGET-WAS phase I/II Clinical Trial: Safety and Clinical Benefit of Lentiviral Hematopoietic Stem Cell Gene Therapy for Wiskott-Aldrich Syndrome

17:10 – 17:25 Dr. Anne Galy, Director of Research Inserm, Genethon, France
Lentiviral Gene Therapy for Wiskott-Aldrich Syndrome: Standardizing the technology.

17:30 – 17:50 - Plenary Session C (XLP and WAS)

Dr. Claire Booth, ICH, London, UK – Dr. Andrew Gennery, Great Northern Children's Hospital, Newcastle upon Tyne, UK**A debate: BMT vs Gene Therapy for non-SCID Immunodeficiencies**

17:50– 18:00 - Open Discussion and Summary
Prof. Bobby Gaspar, Prof. Adrian Thrasher, UCL Institute of Child Health, London, UK

18:00 – 19:00 – 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.

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XLP research trust
Funding medical research into this Silent Killer

Wiskott-Aldrich foundation

The Wiskott Aldrich Syndrome Association (R.A)

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