**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

#### 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 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

#### 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.

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### 15:30 – 15:45 – Short Break

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### 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 a/b 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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