



The 4th International Symposium for  
Researchers and Clinicians

On Wiskott Aldrich Syndrome

MILAN, ITALY  
November 18  
2024

[www.was2024.org](http://www.was2024.org)

## **Agenda**

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

#### **09:00 – 09:10 - Introduction**

Sumathi Iyengar & Amir Kedar, WAS organizations and Anna Villa, IRCCS Ospedale San Raffaele, Milan, Italy.

#### **09:10 – 09:20 – Families Story**

Family story – Gene Therapy – Valentina Pagani, Rosa Giglia.

### **09:20 – 09:50 - Plenary Session A**

**Alessandro Aiuti** - head of the Pediatric Immunohematology Unit at the IRCCS San Raffaele, Milano, Italy.

**“The long path of gene therapy for Wiskott-Aldrich Syndrome”**

#### **09:50 – 10:35 – Gene Therapy Session**

**Chair: Fabio Candotti**

**09:50 - 10:05 Francesca Ferrua** - San Raffaele Telethon Institute for Gene Therapy, Italy.

**Safety and efficacy of lentiviral hematopoietic stem cell Gene Therapy for Wiskott-Aldrich Syndrome**

**10:05 - 10:20 Maria Carmina Castiello** - San Raffaele Telethon Institute for Gene Therapy, Italy.

**Long-term efficacy of lentiviral vector-mediated gene therapy in restoring B-cell homeostasis in Wiskott-Aldrich syndrome patients**

**10:20 - 10:35 Michaela Semeraro** - Centre d'Investigations Cliniques, INSERM CIC1419, Hôpital Necker Enfants malades - Université de Paris-Cité

**Long-term Inflammatory manifestations post lentiviral hematopoietic stem/progenitor cell gene Therapy for Wiskott Aldrich syndrome**

#### **10:35 – 11:05– Gene Therapy Panel**

**Chair: Emma Morris**

Participants: **Alessandro Aiuti, Marina Cavazzana, Luigi Notarangelo, Suhag Parikh.**

**“Lentiviral Gene Therapy for WAS, why is there variability in outcome and how we can address it?”**

### **11:05 – 11:20 – Break**

### **11:20 – 11:50 - Plenary Session B**

**Alessia Cavazza** - Infection, Immunity & Inflammation Department, UCL Institute of child health, London, UK.

**Development of a safe and efficient CRISPR/Cas gene editing platform for the treatment of Wiskott-Aldrich Syndrome**

#### **11:50 – 12:00 – Families Story**

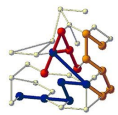
Young Adult Family story – Emily Kinsel

### **12:00 – 12:40 –Clinical International Session I**

**Chair: Anders Fasth**

**12:00 - 12:10 Carmem Bonfim**- Pediatric Transplant and Cellular Therapy Program - Duke University, NC, USA.

**Haploidentical cell transplantation using post-transplantation cyclophosphamide for patients with Wiskott-Aldrich: Excellent outcomes and emerging challenges**



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**12:10 - 12:20 Deepti Suri** - Department of Pediatrics, Postgraduate Institute of Medical Education and Research, Chandigarh, India..

**Correlation of Clinical Profiles, Immune Cells, Genotypes, and Regulatory Gene Expression in Indian Patients with XLT/WAS**

**12:20 - 12:30 Anahita Razaghian** - IAARI and Hakim children hospital, Teheran, Iran.

**Clinical features and outcomes of 39 Wiskott-Aldrich syndrome patients from a single center of Iran**

**12:30 - 12:40 Questions and answers and discussion**

### **12:40 – 13:10 - Plenary Session C**

**Moderator: Luigi Notarangelo** - Immune Deficiency Genetics Section, National Institute of Health, USA.

Participants: **Alessandro Aiuti, Michael Albert, Fabio Candotti, Sumathi Iyengar, Emma Morris**

**“Evolving aspects of WAS: milder forms of the disease and carrier status – from disease perception to clinical implications”**

### **13:10 – 14:10 – Lunch**

### **14:10 – 15:10 –Clinical Session II**

**Chair: Carmem Bonfim**

**14:10 - 14:30 Emma Morris** – **Invited speaker**, Director, UCL Division of Infection and Immunity, London, UK.

**“Allogeneic HSCT for adults with WAS”**

**14:30 - 14:45 Tanja C. Vallée** - Stem cell transplantation unit, Dr. von Haunersches University Children’s Hospital, Munich  
**Allogeneic HSCT with adjusted busulfan dosing results in excellent survival and cure rates for WAS**

**14:45 - 15:00 Yoji Sasahara** – Dep. of Pediatrics & Pediatric Oncology, Tohoku University Graduate School of Medicine

**Efficacy of rituximab for the treatment and prevention of autoimmunity in patients with WAS and XLT**

**15:00 - 15:10 Questions and answers and discussion**

### **15:10 – 15:40 –Clinical Session III**

**Chair: Alessandro Aiuti**

**15:10 - 15:25 Maddalena Migliavacca** - Pediatric Immunohematology and Bone Marrow Transplantation Unit, IRCCS San Raffaele Scientific Institute, Milan, Italy.

**New insights into Wiskott Aldrich Syndrome carriers: A multicentric observation**

**15:25 - 15:40 Markus Walz** - Associate Partner, Magnolia Innovation, New Jersey, USA.

**An International Study of Patient and Caregiver-Reported Burden and Quality of Life in Wiskott-Aldrich Syndrome**

**15:40 - 15:45 Questions and answers and discussion**

### **15:45 – 16:20 – Clinical Session Panel**

**Chair: Michael Albert**

Participants: **Alessandro Aiuti, Siobhan Burns, Fabio Candotti, Luigi Notarangelo**

**'Towards a consensus statement in the management of WAS'**



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### **16:20– 16:35 – Break**

#### **16:35 – 17:10 – Research Session I**

**Chair: Anna Villa**

**16:35 - 16:50 Rhaissa Vieira** - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.  
**Therapeutic Candidate for Enhancing Platelet Production and Function in Wiskott-Aldrich Syndrome and X-Linked Thrombocytopenia**

**16:50 - 17:05 Lia Pinho** - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.  
**From Structure to Function: Understanding the impact of WASp Mutations on Actin Dynamics and T Cell Activation**

**17:05 - 17:10 Questions & answers and discussion**

#### **17:10 – 18:20 - Research Session II**

**Chair: Lisa Westerberg**

**17:10 - 17:30 Yatin Vyas** – Invited speaker, Professor and Chair, Department of Pediatrics, Penn State Health Children's Hospital, USA.

**Functional pairing of WASp and Fanconi anemia proteins in the cell nucleus- co-opting cosmic quantum entanglement**

**17:30 - 17:45 Roberto Amadio** - Cellular Immunology department, University of Trieste and ICGEB Trieste, Italy.  
**Actin polymerization by WASp limits nuclear envelope rupture and inflammation in macrophages**

**17:45 - 18:00 Jordan Chill** - Department of Chemistry, Bar Ilan University, Israel.

**Curation of a Structural WAS/XLT Database: Challenges and First Conclusions**

**18:00 - 18:15 Gianluca Baldanzi** - Department of Translational Medicine, University of Piemonte Orientale, Novara, Italy.  
**DGKα is a negative regulator of megakaryopoiesis inhibited by WASp**

**18:15 - 18:20 Questions & answers and discussion**

#### **18:20 – 18:30 – Closing and Summary of the day**

**Anna Villa, Fabio Candotti, Sumathi Iyengar, Amir Kedar**

### **18:30 – 19:15 – 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 attend.*



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