

# WAS2024

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 - TBA

### **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 – 11:05 - Research Session I**

**Chair: Lisa Westerberg**

**09:50 - 10:10 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**

**10:10 - 10:25 Roberto Amadio** - Cellular Immunology department, University of Trieste and ICGEB Trieste, Italy.

**Actin polymerization by WASp limits nuclear envelope rupture and inflammation in macrophages**

**10:25 - 10:40 Jordan Chill** - Department of Chemistry, Bar Ilan University, Israel.

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

**10:40 - 10:55 Gianluca Baldanzi** - Department of Translational Medicine, University of Piemonte Orientale, Novara, Italy.

**DGKα is a negative regulator of megakaryopoiesis inhibited by WASp**

**10:55 - 11:05 Questions & answers and discussion**

### **11:05 – 11:20 – Break**

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

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

**“Wiskott-Aldrich syndrome – Where do we stand? Where should we go?”**

#### **11:50 – 12:30 – Research Session II**

**Chair: Anna Villa**

**11:50 - 12:05 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**

**12:05 - 12:20 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**

**12:20 - 12:30 Questions & answers and discussion**



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### **12:30 – 13:00 – Clinical International Session I**

**Chair: Anders Fasth**

**12:30 - 12:40 Nguyen Lien Anh Phan** - Children's hospital 1, immuno-hematology Department, Hồ Chí Minh, Vietnam.  
**Genotype-Phenotype Correlation in Wiskott-Aldrich Syndrome: A Study of WAS Gene Mutations in Vietnamese Patients**

**12:40 - 12:50 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**

**12:50 - 13:00 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**

### **13:00 – 13:30 - Plenary Session C**

**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**

### **13:30 – 14:30 – Lunch**

### **14:30 – 15:15 – Gene Therapy Session**

**Chair: Fabio Candotti**

**14:30 - 14:45 Francesca Ferrua** - San Raffaele Telethon Institute for Gene Therapy, Italy.  
**Safety and efficacy of lentiviral hematopoietic stem cell Gene Therapy for Wiskott-Aldrich Syndrome**

**14:45 - 15:00 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**

**15:00 - 15:15 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**

### **15:15 – 15:45– Gene Therapy Panel**

**Chair: Emma Morris**

Participants: TBA

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

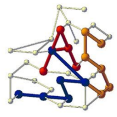
### **15:45 – 16:45 – Clinical Session II**

**Chair: Carmem Bonfim**

**15:45 - 16:05 Emma Morris** – Invited speaker, Director, UCL Division of Infection and Immunity, London, UK.  
**"Allogeneic HSCT for adults with WAS"**

**16:05 - 16:20 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**

**16:20 - 16:35 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**



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16:35 - 16:45 Questions and answers and discussion

**16:45 – 16:55 – Families Story**

Young Adult Family story - TBA

**16:55 – 17:15 – Break**

**17:15 – 17:50 – Clinical Session III**

**Chair: Alessandro Aiuti**

**17:15 - 17:30 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**

**17:30 - 17:45 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**

17:45 - 17:50 Questions and answers and discussion

**17:50 – 18:20 – Clinical Session Panel**

**Chair: Michael Albert**

Participants: TBA

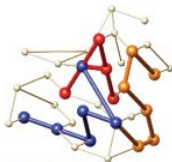
**'Towards a consensus statement in the management of WAS'**

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

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

**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.



The Wiskott Aldrich Syndrome Association (R.A)

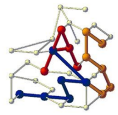


Wiskott-Aldrich  
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