



The 4th International Symposium for
Researchers and Clinicians
On Wiskott Aldrich Syndrome

MILAN, ITALY
November 18
2024

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.

Awaiting presentation title

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.

Awaiting presentation title

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.

DGK α 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

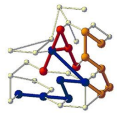
11:50 – 12:05 Rhaissa Vieira - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.

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 Jean-Sebastien Diana - Biotherapy Department, Necker Hospital, Pros, France.
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

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

15:45 – 16:45 – Clinical Session II

Chair: Luigi Notarangelo

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

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



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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 Alexandra Wilds - 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

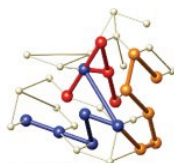
'Towards a consensus statement in the management of WAS'

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
foundation



Jeffrey Modell
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Curing PI. Worldwide.

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