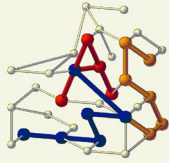


WAS2022



June 17
2022
Munich, Germany

THE 3rd INTERNATIONAL
SYMPOSIUM FOR RESEARCHERS
AND CLINICIANS

ON WISKOTT ALDRICH SYNDROME

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SAVE THE DATE

Agenda

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

09:00 – 09:15 - Introduction

Sumathi Iyengar- Amir Kedar, WAS organizations and Michael Albert, LMU, München, Germany

09:15 – 09:45 - Plenary Session A

Anna Villa, San Raffaele Telethon Institute for Gene Therapy, Milano & IRGB, National Research Council
Platelets defects in Wiskott-Aldrich Syndrome

09:45 – 10:45 - Research Session I

Chair: Adrian Thrasher

09:45 - 10:00 Rhaissa Vieira - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.

Flow Cytometry-based Drug Screening for stabilization of WASp to restore Megakaryocyte and Platelet Function

10:00 - 10:15 Jordan Chill - Department of Chemistry, Bar Ilan University, Israel.

Towards a Molecular Understanding of WAS/XLT Using Biological Nuclear Magnetic Resonance

10:15 - 10:30 Roberta D'aulerio - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.

Revealing the molecular role of WASp in the nucleus of B cells.

10:30 - 10:45 Questions & answers and discussion

10:45 – 11:05 – Break

11:05 – 11:35 - Plenary Session B

David J. Rawlings - Director, Center for immunity and immunotherapies, Seattle children's research institute, USA.

“Lessons learned regarding immune tolerance and progress towards new therapies for WAS”

11:35 – 12:15 –Research Session II

Chair: Lisa Westerberg

11:35 - 11:50 Minghui He - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.

Constitutively active WASp in X-linked neutropenia leads to compromised B cell division and accelerated plasma cell differentiation

11:50 - 12:05 Lia Pinho - Department of Microbiology Tumor and Cell Biology, Karolinska Institutet, Sweden.

A novel Arg431Trp mutation of WASp causes an intermediate WAS and XLN phenotype

12:05 - 12:15 Questions & answers and discussion

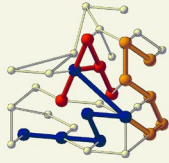
12:15 – 12:45 – Gene Therapy Session

Chair: Anna Villa

12:15 - 12:45 Francesca Ferrua - San Raffaele Telethon Institute for Gene Therapy, Italy.

Hematopoietic Stem and Progenitor Cell Lentiviral Gene Therapy for Wiskott-Aldrich Syndrome: Up to 10.5 Years of Follow-Up in 17 Subjects

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12:45 – 13:45 - Lunch

13:45 – 14:50 – Gene Therapy Session (continued)

Chair: Anna Villa

13:45 - 14:05 Ryan Wong (Invited speaker) – ImmunoVec, CA, USA.

Bioinformatic-Guided Design of a Lentiviral Vector for Wiskott-Aldrich Syndrome Recapitulates Endogenous WAS Gene Expression

14:05 - 14:20 Maria Pia Cicalese - Clinical Research Unit (CRU), IRCCS San Raffaele Scientific Institute, Milan, Italy.
Restoration of follicular T cells in patients with Wiskott-Aldrich Syndrome after gene therapy

14:20 – 14:35 Melissa Pille – Department of Diagnostic Sciences, Ghent University, Belgium.
CRISPR/Cas9- mediated gene editing for treatment of the Wiskott-Aldrich Syndrome

14:35 - 14:50 Questions & answers and discussion

14:50 – 15:50 –Clinical Session I

Chair: Fabio Candotti

14:50 - 15:05 Annarosa Soresina – Pediatrics Clinic, ASST Spedali Civili of Brescia, Univ. of Brescia, Italy.

Long term outcome in Wiskott- Aldrich Syndrome (WAS) and X-Linked Thrombocytopenia (XLT): a prospective multicenter national study.

15:05 - 15:20 Deepti Suri – Allergy and Immunology Unit, Department of Pediatrics, Postgraduate Institute of Medical Education and Research, Chandigarh, India

Wiskott Aldrich syndrome: 15 years' experience at Post Graduate Institute of Medical Education and Research, Chandigarh, India.

15:20 - 15:35 Charline Miot- University Hospital of Angers, France.

Long-term Outcome of Mild WAS/XLT Patients: Experience from the Registry of the French National Reference Center for Primary Immunodeficiencies (CEREDIH).

15:35 - 15:50 Questions and answers and discussion

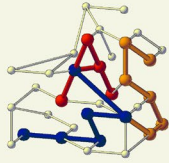
15:50 – 16:10 –Break

16:10 – 16:40 - Plenary Session C

Michael Albert, Head of the pediatric stem cell transplantation unit at the Dr. von Hauner Children's Hospital, LMU Munich

HSCT for WAS – What have we learned in 50 years and what promises does the future hold?

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16:40 – 17:40 – Clinical Session II

Chair: Michael Albert

16:40 - 17:00 Carmem Bonfim (Invited speaker) – Director Pediatric Blood and Marrow Transplantation Program, Federal University of Paraná, Curitiba, Brazil.

The challenges and hurdles to improve outcomes after HSCT for patients with Wiskott Aldrich syndrome in restricted resources countries

17:00 - 17:15 Samuele Naviglio - Department of Pediatric Hematology-Oncology, Institute for Maternal and Child Health IRCCS "Burlo Garofolo", Trieste, Italy.

Autoinflammatory manifestations and response to treatment with anti-interleukin-1 agents in patients with Wiskott-Aldrich Syndrome

17:15 - 17:30 Dr. Suhag Parikh – Emory University, Department of Pediatrics, Aflac Cancer and Blood Disorders Center, Children's Healthcare of Atlanta

Clinical spectrum of Carriers of X-linked Wiskott Aldrich syndrome gene: Self-reported survey of 193 carriers

17:30 - 17:45 Questions and answers and discussion

17:45– 18:00 – Discussion and Summary of the day

Adrian Trasher, Michael Albert

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.



The Wiskott Aldrich Syndrome Association (R.A)



Wiskott-Aldrich
foundation



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