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: Lisa Westerberg

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: David J. Rawlings

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 – 13:00 – Gene Therapy Session
Chair: Anna Villa

12:15 - 12:35 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

12:35 - 12:45 Maria Carmina Castiello - San Raffaele Telethon Institute for Gene Therapy, IRGB National Research Council, Italy.

B cell reconstitution in WAS patients after Gene Therapy

12:45 - 13:00 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

13:00 – 14:00 - Lunch

14:00 – 14:45– Gene Therapy Session (continued)

Chair: Anna Villa

14:00 - 14:20 Ryan Wong (Invited speaker) – ImmunoVec, CA, USA.

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

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 - 14:45 Questions & answers and discussion

14:45 – 15:15– Gene Therapy Panel

Chair: Michael Albert

Anne Galy, Fabio Candotti, Anna Villa, David Rawlings, Francesca Ferrua, Suhag Parikh

"Future of Gene Therapy for WAS: Challenges and Opportunities"

15:15 – 16:10 –Clinical Session I

Chair: Fabio Candotti

15:15 - 15:30 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.

15:30 - 15:45 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.

15:45 - 16:00 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).

16:00 - 16:10 Questions and answers and discussion

16:10 – 16:25 –Break

16:25 – 16:55 - 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?

16:55 – 17:55 –Clinical Session II
Chair: ***Michael Albert***

16:55 - 17:15 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

17:15 - 17:30 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

17:30 - 17:45 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

17:45 - 17:55 Questions and answers and discussion

17:55– 18:10 – Closing and Summary of the day
Michael Albert, Fabio Candotti, Francesca Ferrua

18:10 – 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

EUROPEAN JOINT PROGRAMME RARE DISEASES

Jeffrey Modell Foundation | Curing PI. Worldwide.

This Networking event has received funding from the European Union's Horizon 2020 research and innovation programme under the EJP RD COFUND-EJP N° 825575

The WAS2022 Symposium was made possible, in part, by a grant provided by the Jeffrey Modell Foundation WIN Program.

Last update 12-06-2022