

RAPID 2024 Program

Recent

Advances in

Primary

Immunodysregulation

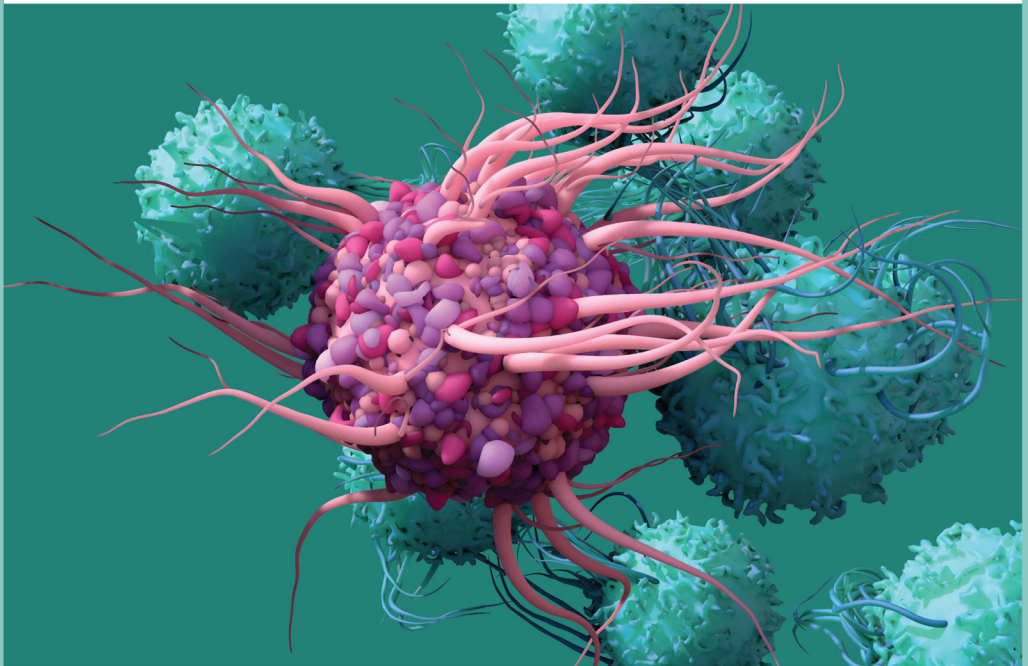
Disorders



HB Auditorium

May 9 & 10, 2024

in collaboration with NIH/NIAID



Recent Advances in Primary Immunodysregulation Disorders (RAPID) Conference

Sidra Medicine, Qatar

May 9 - 10, 2024

Program

Day 1:

Thursday, May 9th

7:30 – 8:30 am Registration

8:30 – 9:00 am Opening Session and Welcome Remarks

9:00 – 10:30 am Session 1:

Primary Immunodeficiency spectrum: What is new

Moderators: Nicholas van Panhuys, Sidra Medicine

Elif Aydiner, Marmara University, Türkiye

- **Overview on What's new in IEI – spectrum & diagnosis**
Kate Sullivan, Childrens' Hospital of Philadelphia
- **Inflammatory bowel disease - gene, mechanisms and treatments**
Holm Uhlig, Oxford University
- **Endosome dysfunction leads to gain-of-function TLR7 and human lupus**
Fabian Hauck, LMU Munich University Hospital
- **An immunodysregulatory syndrome caused by aberrant G-protein signaling**
Helen Su, NIAID/NIH

10:30 – 11:00 am Coffee break

11:00 am – Abstract Presentations on the PID spectrum

12:15 pm Moderators: Waleed Al-Herz, Kuwait University

Aisha Elmarsafy, Cairo University

Best abstracts will be selected for oral presentation

Day 1:

Thursday, May 9th

12:15 – 1:30 pm Lunch

Career Development Panel Session

*Moderators: Deena Wafadari, Mohammed Bin Rashid
University, UAE*

Rafah Mackeh, Sidra Medicine

Panelists:

- Medhat Askar, Associate Vice President for Clinical Affairs and Professor of Immunology at Qatar University
- Nayef Mazloum, Assistant Dean for Student Research at WCM-Q and Assistant Professor of Microbiology and Immunology at WCM-Q and HBKU
- Suleiman Al-Hammadi, Dean of College of Medicine, Mohammed Bin Rashid University, UAE
- Samar Al Emadi, Chief of Rheumatology Division and Director of Rheumatology Fellowship Program, HMC
- Maryam Al-Nesf, Head of Allergy & Immunology Section and Allergy & Immunology Program Director, HMC
- Ibrahim Janahi, Chair of Medical Education and Division Chief of Pulmonology at Sidra Medicine

1:30 – 3:30 pm

**Diagnostic challenges in Inborn errors of immunity
(Primary Immunodeficiency)**

*Moderators: Suleiman Al-Hammadi, Mohammed Bin Rashid
University, UAE*

*Michel Massaad, American University of Beirut,
Lebanon*

- **The Ever Changing Genetic Landscape of IEI**
Jolan Walter, University of South Florida
- **Genetics of IEI in the MENA region**
Hamoud Almousa, King Faisal Specialist Hospital
& Research Center

Day 1:

Thursday, May 9th

- **Importance of functional investigations of Variants of Uncertain Significance (VUS)**
Amel Hassan, Qatar University
- **The subtleties of JAK-STAT biology within the immune system in health and disease**
Raphaela Goldbach-Mansky, NIAID/NIH
- **Rheumatological complications of primary immune dysregulation**
Raphaela Goldbach-Mansky, NIAID/NIH

3:30 – 4:00 pm Coffee break

**4:00 – 5:15 pm Genetic Screening for IEI in the MENA region -
Panel discussions**

*Moderators: Bandar Al-Saud, King Faisal Specialist
Hospital and Research Center, KSA
Bernice Lo, Sidra Medicine*

Introductory 15-minute talk: *Donald Love, Sidra Medicine*

Panelists to address the following discussion questions:

- Subcontracting or Developing institutional capacities?
- What strategy for screening: WES or gene panels?
- Bioinformatics capacities to be developed locally or not?
- Integrating AI with bioinformatics

Panelists:

- *Anas Alazami, King Faisal Specialist Hospital and Research Centre*
- *Imen Ben Mustapha, Pasteur Institute of Tunis*
- *Serdar Ceylaner, Lokman Hekim Üniversitesi*
- *Wangling Yang, The University of Hong Kong*
- *Donald Love, Sidra Medicine*

Day 2:

Friday, May 10th

8:00 – 10:00 am **Clinical Presentations of Organ-based Immune Dysregulation**

Moderators: Gehad Elghazali, UAE University

Sharon Bout-Tabaku, Sidra Medicine

- **Clinical features of monogenic immunodysregulation diseases from the Middle East and North Africa registry**
Hassan Abolhassani, Tehran University of Medical Sciences & Karolinska Institutet
- **Allergic and cutaneous presentations in patients with primary immunodysregulation disorders**
Ridha Barbouche, Arabian Gulf University
- **Malignancy as a presenting feature or complication of primary immunodysregulation**
Nima Rezaei, Research Center for Immunodeficiencies, Children's Medical Center, Tehran University of Medical Sciences
- **Inflammatory bowel disease: clinical pearls for monogenic vs. polygenic presentation and prognosis**
Mamoun Elawad, Sidra Medicine
- **The frontiers between hypogammaglobulinemia and immune dysregulation among the spectrum of CVID patients**
Ghassan Dbaibo, American University of Beirut

10:00 – 10:30 am **Coffee break**

10:30 – 11:30 am **Abstract Presentations on clinical manifestations, diagnostics, and therapeutics of immune dysregulation**

Moderators: Ibtihal Benhsaien, King Hassan II University, Morocco

Buthaina Al-Adba, Sidra Medicine

Best abstracts will be selected for oral presentation

Day 2:

Friday, May 10th

11:30 am – Lunch / Jumma prayer

12:30 pm

Sponsor Workshop: Biotest (starting 12:00 pm)

Mohammed Yousuf Karim, Sidra Medicine

Case presentation: Clinical pearls to distinguish IEI from secondary immunodeficiency

12:30 – 2:00 pm Immune Dysregulation Disorders: Emerging Therapeutic Strategies and Modalities

Moderators: Mohammed Yousuf Karim, Sidra Medicine

Deniz Çağdaş Ayvaz, Hacettepe University, Türkiye

- **Vicki Modell Memorial Lecture**

Updates from the Primary Immune Deficiency Treatment Consortium (PIDTC)

Jennifer Puck, University of California – San Francisco

- **Leniolisib for APDS/PASLI Disease**

Mike Lenardo, NIAID/NIH

- **Complement Inhibitor Therapy for CHAPLE Disease**

Ahmet Özen, Marmara University

2:00 – 3:00 pm Poster Session

3:00 – 4:00 pm Recent Advances in Corrective Therapies for Inborn Errors of Immunity (IEI)

Moderators: Aydan Ikinioğulları, Ankara University Medical School, Türkiye

Raed Alzyoud, Queen Rania Children's Hospital, Jordan

- **Gene Therapy for Inborn Errors of Immunity: Current Progress and Prospects**

Maria Pia Cicalese, San Raffaele Telethon Institute for Gene Therapy

Day 2:

Friday, May 10th

- **Lentivirus gene therapy trial for Artemis (DCLRE1C) deficient SCID**
Jennifer Puck, University of California – San Francisco
- **Expanding the Scope of HSCT in the Treatment of IEIs Beyond SCID**
Danielle Arnold, Center for Cancer Research/NCI/NIH

4:00 – 5:30 pm

Viewpoint Topics

Curative therapies vs. pharmacotherapy in inborn errors of immunity

*Moderators: Brahim Belaid, University of Algiers 1, Algeria
Ihsan Gursel, Bilkent University, Türkiye*

Topic 1:

LRBA Deficiency/CTLA4 Haploinsufficiency Disorders

- The latest advancements, benefits, and limitations of pharmacotherapy.
Safa Baris, Marmara University, School of Medicine
- The effectiveness, challenges, and potential of Hematopoietic Stem Cell Transplantation (HSCT).
Figen Doğu, Ankara University, Faculty of Medicine

Topic 2:

Chronic Granulomatous Disease (CGD)

- Insights into pharmacological interventions, their impact, and scope.
Jennifer W. Leiding, Johns Hopkins University
- HSCT and emerging gene therapy options: when to consider curative therapies in CGD.
Danielle Arnold, National Cancer Institute/NIH

5:30 – 6:00 pm

Wrap-up and closing

