Conference Agenda

Educational objectives

- Describe the available somatic cell therapies: development phases, clinical trials, and regulatory aspects;
- Describe the available gene therapies: development phases, clinical trials, and regulatory aspects;
- Describe the available molecular targeted therapies: development phases, clinical trials, and regulatory aspects;
- Discuss advanced therapeutic and diagnostic technologies in diabetes, surgery, etc.

Saturday 28 October 2017

<table>
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<tr>
<th>Time</th>
<th>Session</th>
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<tr>
<td>7:00-8:00</td>
<td>Registration and welcome drinks</td>
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<td>8:00-8:10</td>
<td>Welcome note</td>
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<td>Ena Wang, A/CRO</td>
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Objectives:
Describe gene therapy traditional approaches
Describe gene therapy novel approaches as gene editing
Describe the available gene therapies for primary immunodeficiency, hematological diseases and haemophilia A

Moderators:
Dr. Sara Deola, Sidra Medical and Research Center, Qatar
Dr. Antonia Follenzi, University of Novara, Italy

8:10 – 8:55
Key note speaker:
Prof. Adrian Thrasher
UCL Great Ormond Street Institute of Child Health- London, UK
“Evolving Gene Therapy for Primary Immunodeficiency”

8:55-9:20
Dr. Matthew Porteus *
Stanford University, California, US
“Genome Editing of Stem Cells to Cure Genetic Diseases of the Blood and Immune System”

*Please note that this presentation is not count towards CME credit
9:20-9:45

**Dr. Waseem Qasim**  
University College London, UK  
“Gene Engineered Immune Therapy”

9:45-10:10

**Dr. Ramsay Fuleihan**  
Sidra Medical Research Center, Qatar  
“Stem cell transplantation (reduced intensity conditioning) and obstacles and new approaches to gene therapy for Hyper IgM syndrome”

10:10– 10:35

**Dr. Antonia Follenzi**  
University of Novara, Italy  
“Cell & Gene Therapy for Hemophilia A”

10:35-10:55  
Coffee break

**Day 1 Session 2: “Cell Therapy and Adoptive Cell Therapy: A Cell for Every Disease”**

**Objectives:**
- Discuss the potential of mesenchymal stromal cells in diabetes and inflammatory gut diseases
- Describe the antigen-specific immunotherapy
- Discuss the potential of iPSC in diabetes treatment

**Moderators:**
- **Prof. Shahrad Taheri**, Weill Cornell Medical College-Qatar  
- **Prof. Khalid Hussain**, Sidra Medical and Research Center, Qatar

10:55-11:20

**Dr. Katarina Le Blanc**  
Karolinska Institutet- Stockholm, Sweden  
“MSC in Clinical Trials for Type 1 Diabetes”

11:20-11:45

**Dr. Maria Ester Bernardo**  
San Raffaele Hospital- Milan, Italy  
“Autologous Bone Marrow-Derived Mesenchymal Stromal Cells in the Treatment of Fistulising Crohn’s disease”

11:45-12:10

**Dr. Patrizia Comoli**
Day 1 Session 3: “Novel Transplant Approaches: Tailoring Cells for Clinical Needs”

**Objectives:**
- Describe novel transplant approaches for diabetes and inflammatory gut diseases
- Describe thymus transplant
- Discuss the potential of beta cells transplant in diabetes

**Moderators:**
- Dr. Qian Zhang, Sidra Medical and Research Center, Qatar
- Dr. Mamoun Elawad, Sidra Medical and Research Center, Qatar

13:30-13:55
**Dr. Lorenzo Piemonti**
IRCCS San Raffaele Scientific Institute-Milan, Italy
“Toward Beta Cell Replacement for Diabetes”

13:55-14:20
**Dr. Peter Parham**
Stanford University- Stanford, California
“HLA & KIR in Human Health and Survival”

14:20-14:45
**Dr. Graham Davies**
Great Ormond Street Hospital- London, UK
“Thymus Transplantation”

14:45-15:10
**Dr. Mamoun Elawad**
Sidra Medical and Research Center- Doha, Qatar
“Allogeneic HSCT for Inflammatory Gut Diseases”
Day 1 Session 4: “Advanced Therapies: Quality and Compliance Issues”

Objectives:
Describe quality and compliance issues for advanced treatments
Describe Jacie accreditation

Moderators:
Mrs. Rim Imam, Sidra Medical and Research Center, Qatar
Mr. Massimino Jan Miele, Sidra Medical and Research Center, Qatar

15:10-15:35
Dr. Amel Hassan
Qatar Foundation- Doha, Qatar
“Hematopoietic Stem Cell Transplant for PID”

15:35-15:55
Coffee break

15:55-16:20
Dr. Fawzi Teskrat
“Inspection of ATMP’s Activities”

16:20-16:45
Mr. Eoin McGrath
Joint Accreditation Committee for EBMT and ISCT
“JACIE Accreditation: an overview”

16:45-17:10
“New therapies: adapting standards and regulations to immune effector cells”

Closing Remarks of Day 1

Sunday 29th October 2017

7:00-8:00
Registration and refreshments

Day 2 Session 1: “Molecular Targeted Therapy: New Weapons Are Available to Increase the Efficacy”

Objectives:
Describe cancer molecular targeted therapies
Describe novel molecular targeted therapies for autoimmune and inflammatory diseases

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<tr>
<th>Time</th>
<th>Speaker</th>
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<tr>
<td>8:00-8:25</td>
<td><strong>Dr. Raya Saab</strong></td>
<td>American University of Beirut, Lebanon</td>
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<td></td>
<td>“Genomics of Childhood Cancer”</td>
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<td>8:25-8:50</td>
<td><strong>Dr. Amar Gajjar</strong></td>
<td>Saint Jude Children Research Hospital, Memphis, US</td>
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<td>“Molecularly Directed Therapy for Pediatric Brain Tumors”</td>
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<td>8:50-9:15</td>
<td><strong>Dr. Massimo Gadina</strong></td>
<td>National Institutes of Health, Bethesda, Maryland, US</td>
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<td>“Inhibition of Cytokine Signaling in Autoimmune and Inflammatory Diseases: The Coming of Age of JAK Inhibitors”</td>
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<td>9:10-9:35</td>
<td><strong>Dr. Holm Uhlig</strong></td>
<td>University of Oxford, UK</td>
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<td>“New Non-Transplant Approach in Treating CGD and Primary Neutropenia”</td>
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**Coffee break**

**Day 2 Session 2: “The Evolution of Immunotherapy for Cancer Treatment: where Are We?”**

**Objectives:**
- Describe cancer immunotherapy
- Discuss the impact of immune checkpoints inhibitors in cancer treatment
- Describe the cancer immunotherapies available in Qatar
Moderators:

Dr. Davide Bedognetti, Sidra Medical and Research Center, Qatar

Dr. Said Dermime, National Center for Cancer Care and Research, Hamad Medical Corporation, Doha, Qatar

Key note speaker:

Dr. Soldano Ferrone
Harvard Medical School, Boston, US
“HLA Antigens and Immunotherapy of Malignant Diseases”

Dr. Michele Maio
Division of Medical Oncology and Immunotherapy
University Hospital of Siena, Siena, Italy
“Immune Checkpoint Inhibitors”

Dr. Francesco M. Marincola
AbbVie Biotherapeutics Corporation-California, US
“Addressing Cancer Responsiveness Immunotherapy”

Dr. Gianfranco Pittari
National Center for Cancer Care and Research, Hamad Medical Corporation, Doha, Qatar
“Translational implications of self-specific NK cell reactivity for AML treatment”

Day 2 Session 3: “CAR-T Cells: Manufacture and Applications of Cancer-Targeted Cell Therapy”

Objectives:

Describe the results of the CAR-T cell treatment in cancer
Discuss the potential of CAR-T cells

Moderators:

Dr. Cristina Maccalli, Sidra Medical and Research Center, Qatar

Dr. Gianfranco Pittari, Hamad General Hospital, Doha, Qatar
Key note speaker:
Dr. Gianpietro Dotti
University of North Carolina at Chapel Hill, US
“Car-T cells: From Bench to Bedside”

12:30-13:30 Lunch break

Dr. Kevin Joseph Curran
Memorial Sloan Kettering, New York, US
“CAR T-Cell for Cancer Immunotherapy”

13:30-13:55

Dr. Stephen Hunger
Children’s Hospital of Philadelphia, Philadelphia, US
“Treatment of Relapsed Pediatric Acute Lymphoblastic Leukemia: The Promise of CAR T-Cell Therapy”

13:55-14:20

Dr. David Stroncek
National Institutes of Health, Bethesda, Maryland, US
“CAR-T Cells: Promise and Problems”

14:20-14:45

Dr. Winfried Wels
Georg Speyer Haus, Institute for Tumor Biology and Experimental Therapy, Frankfurt, Germany
“CAR-engineered NK cell for adoptive cancer immunotherapy”

14:45-15:10

Coffee break

15:10-15:30

15.30 – 16.00 Panel Discussion

Day 2 Session 4: “SIDRA at the forefront of Advanced Technologies”

Objectives:
Describe the advanced technologies available at Sidra in the field of diabetes and surgery

Moderator:
Prof. Khalid Hussain, Sidra Medical and Research Center, Qatar
Prof. Ziyad Hijazi, Sidra Medical and Research Center, Qatar

16:00 – 16:25

Prof. Ziyad Hijazi
Sidra Medical and Research Center – Doha, Qatar
“Current State of Percutaneous Pulmonary Valve Replacement”

Dr. Goran Petrovski
Sidra Medical and Research Center – Doha, Qatar
“Closing the loop in Diabetes: The impact of Sensor Augmented Pump”

Dr. Abdalla Zarroug
Sidra Medical and Research Center – Doha, Qatar
“Fetal Surgery”

Student Corner

17:15- 17:25

Khalifa Ahmed Elmagarmid
“Repurposing rheumatoid arthritis drug auranofin for intestinal decolonization of vancomycin-resistant enterococci”

Closing Remarks