Friday, November 13, 2020

09:30–09:40 Welcome

SESSION I – STEM CELL BIOLOGY: FROM STRESS TO FAILURE
Chairs: Tim Brümmendorf and Markus Manz

09:40-09:45 Introduction Tim Brümmendorf & Markus Manz
09:45-10:00 Haemopoietic stem cell activation and failure Andreas Trumpp (Heidelberg)
10:00-10:15 Genotoxic stress and repair in stem cells Ketan J. Patel (Cambridge)
10:15-10:30 Inflammageing of the haemopoietic system Markus Manz (Zürich)

SELECTED BRIEF ORAL COMMUNICATION

10:30-10:40 Immunologic Basis to Poor Graft Function Post Allogeneic Transplantation. Ashvind Prabahran (Melbourne)
10:40-11:10 Discussion

11:10-11:20 Coffee Break

SESSION II – CONSTITUTIONAL MARROW FAILURE 1: FANCONI ANAEMIA (FA)
Chairs: Carlo Dufour and Inderjeet Dokal

11:20-11:25 Introduction Carlo Dufour & Inderjeet Dokal
LIVE Programme
CET Time

11:25-11:40 Fanconi anaemia : diagnostic and natural history in the bone marrow
Jean Soulier (Paris)

11:40-11:55 Classical and new treatment of Fanconi anaemia
Carlo Dufour (Genova)

11:55-12:10 Gene therapy for Fanconi anaemia
Juan Bueren (Madrid)

SELECTED BRIEF ORAL COMMUNICATION

12:10-12:20 Genetic Rescue of Fanca-/- HSPCs with Evi-1 Overexpression Suggests a Mechanistic Link with DNA Damage Response
Susanne Lux (Heidelberg)

12:20-12:50 Discussion

SESSION III – CONSTITUTIONAL MARROW FAILURE 2: RIBOSOME DISEASES
Chairs: Marc H. G. P. Raaijmakers and Alan Warren

14:00-14:05 Introduction
Marc H.G.P. Raaijmakers & Alan Warren

14:05-14:20 Diamond-Blackfan anaemia (DBA), ribosomes and haemopoiesis
Vijay Sankaran (Boston)

14:20-14:35 Shwachman-Diamond syndrome (SDS) and related ribosomopathies
Alan Warren (Cambridge)

14:35-14:50 Management of ribosomopathies
Yigal Dror (Toronto)

SELECTED BRIEF ORAL COMMUNICATION

14:50-15:00 A Single Base Change Makes the Cure: Correcting the Most Common SBDS Mutation in Shwachman-Diamond Syndrome
Chi-Yuan Zhang (Boston)

15:00-15:30 Discussion

SESSION IV – CONSTITUTIONAL MARROW FAILURE 3: TELOMERE BIOLOGY DISEASES
Chairs: Inderjeet Dokal and Tim Brümmendorf

15:40-15:45 Introduction
Inderjeet Dokal & Tim Brümmendorf

15:45-16:00 Telomeres and telomerase in health and ageing
Alison Bertuch (Houston)

16:00-16:15 Dyskeratosis congenita and related haematological diseases
Inderjeet Dokal (London)

16:15-16:30 Diagnosis and clinical presentation of adult onset telomeropathies
Fabian Beier (Aachen)
SELECTED BRIEF ORAL COMMUNICATION

16:30-16:40 Natural History and Outcomes Depending on Organ System Involvement in Patients with Clinically-Relevant Short Telomeres  
Abhishek Mangaonkar (Rochester)

16:40-17:10 Discussion

17:15-18:15 Poster walks
Leaders: Tim Brümmendorf and Marc H.G.P. Raaijmakers

Saturday, November 14, 2020

12:00-13:00 3 Simultaneous Meet the Expert sessions

1. Role of Next Generation Sequencing in the diagnosis and follow-up of patients with BMF  
   Marc H.G.P. Raaijmakers & Jean Soulier

2. Management of challenging situations in PNH  
   Britta Höchsmann & Rosario Notaro

3. How to properly diagnose and treat telomere biology disorders in children and adults  
   Tim Brümmendorf & Inderjeet Dokal

13:05-14:05 SATELLITE SYMPOSIUM:

Title: Beyond current endpoints: a patient-centric approach to PNH treatment
Chair: Régis Peffault de Latour
Speakers: Pascale Burmester, Talha Munir and Jens Panse

This symposium is organized by Roche

SESSION V – IMMUNE APLASTIC ANAEMIA AND PAROXYSMAL NOCTURNAL HAEMOGLOBINURIA (PNH) 1. PATHOPHYSIOLOGY AND DIAGNOSIS
Chairs: Neal Young and Regis Peffault de Latour

14:10-14:15 Introduction  
Neal Young & Regis Peffault de Latour

14:15-14:30 Pathophysiology of immune aplastic anaemia  
Neal Young (Bethesda)

14:30-14:45 Pathophysiology of PNH  
Rosario Notaro (Florence)

14:45-15:00 Modern diagnosis of aplastic anaemia and PNH  
Austin Kulasekararaj (London)
SELECTED BRIEF ORAL COMMUNICATION

15:00-15:10  Single Cell eQTL Mapping Identifies Cell-Type Specific Control of Autoimmune Disease  
Kirsten Fairfax (Hobart)

15:10-15:35  Discussion

15:40-16:40  SATELLITE SYMPOSIUM:

Title: A journey through the SAA and PNH treatment landscapes
Chair: Régis Peffault de Latour and Antonio Risitano
Speakers: Régis Peffault de Latour and Antonio Risitano

This symposium is organized by Novartis

SESSION VI – IDIOPATHIC APLASTIC ANAEMIA AND PAROXYSMAL NOCTURAL HAEMOGLOBINURIA (PNH) 2. DIFFERENTIAL DIAGNOSIS AND CLONAL EVOLUTION
Chairs: Neal Young and Jaroslaw P. Maciejewski

16:45-16:50  Introduction  Neal Young & Jaroslaw P. Maciejewski

16:50-17:20  Keynote Presentation:
Bone marrow failure and somatic mutations: the PNH paradigm  
Lucio Luzzatto (Dar-es-Salaam)

17:20-17:30  Discussion

17:30-17:45  Innate immunity and clonal evolution in bone marrow failure  
Alan List (Tampa)

17:45-18:00  Impact of clonal haemopoiesis in aplastic anaemia  
Jaroslaw P. Maciejewski (Cleveland)

18:00-18:15  Health-related quality of life and its assessment in aplastic anaemia and paroxysmal nocturnal haemoglobinuria  
Jens Panse (Aachen)

SELECTED BRIEF ORAL COMMUNICATION

18:15-18:25  The Metabolomic Status of the Differentiating Myeloid Lineage in MDS with Low and High Bone Marrow Blast Counts.  
Aikaterini Poulaki (Athens)

18:25-18:55  Discussion

19:00-20:00  3 Simultaneous Meet the Expert Sessions

4. Source and sequence of BMT in acquired aplastic anaemia  Andrea Bacigalupo & Neal Young
5. When and how to do BMT in inherited bone marrow failure syndromes?  Yigal Dror & Carlo Dufour
SESSION VII – TREATMENT OF IMMUNE APLASTIC ANAEMIA (AA)
Chairs: Andrea Bacigalupo and Carlo Dufour

14:05-14:10 Introduction Andrea Bacigalupo & Carlo Dufour

14:10-14:25 Immunosuppression and TPO agonists Régis Peffault de Latour (Paris)

14:25-14:40 Transplantation for acquired SAA: are we making progress? Andrea Bacigalupo (Rome)

14:40-14:55 How to handle moderate aplastic anaemia Britta Höchsmann (Ulm)

SELECTED BRIEF ORAL COMMUNICATION

14:55-15:05 Blockade of Common Gamma Chain Cytokines with REGN7257, an Interleukin 2 Receptor Gamma (IL2RG) Monoclonal Antibody, Protected Mice from T Cell-Mediated Disease Audrey Le Floc’h (Tarrytown)

15:05-15:35 Discussion

SESSION VIII – TREATMENT OF PAROXYSMAL NOCTURNAL HAEMOGLOBINURIA (PNH)
Chairs: Regis Peffault de Latour and Hubert Schrezenmeier

15:35-15:40 Introduction Regis Peffault de Latour & Hubert Schrezenmeier

15:40-15:55 Classical treatment of PNH Hubert Schrezenmeier (Ulm)

15:55-16:10 Challenging situations Peter Hillmen (Leeds)

16:10-16:25 Novel complement inhibitors Antonio Risitano (Naples)

SELECTED BRIEF ORAL COMMUNICATION

16:25-16:35 Results of the Pegasus Phase 3 Randomized Trial Demonstrating Superiority of the C3 Inhibitor, Pegcetacoplan, Compared to Eculizumab in Patients with Paroxysmal Nocturnal Hemoglobinuria Regis Peffault de Latour (Paris)
SESSION IX – EMERGING AREAS AND NEW CHALLENGES
Chairs: Marc H. G. P. Raaijmakers and Lucy Godley

17:15-17:20 Introduction
Marc H.G.P. Raaijmakers & Lucy Godley

17:20-17:35 Genetic editing of haemopoietic stem cells.
Are we getting there?
Matthew Porteus (Stanford)

17:35-17:50 MDS Stem Cell Surveillance, Characterization and Therapeutic Targeting
Sten Eirik Jacobsen (Stockholm)

17:50-18:05 Genetic predisposition for myeloid malignancies:
clinical management
Lucy Godley (Chicago)

SELECTED BRIEF ORAL COMMUNICATION

18:05-18:15 Prevalence of Inherited Predisposition Syndromes in Young Patients with Acute Myeloid Leukemia and Aberrant Karyotypes
Fabian Beier (Aachen)

18:15-18:45 Discussion

18:45-19:15 Closing Remarks

MEETING CLOSURE