VIRTUAL CONFERENCE
Translational Research Conference

BONE MARROW FAILURE DISORDERS:
From the cell to the cure of the disease

November 13-15, 2020
Chairpersons: Tim Brümmendorf, Inderjeet Dokal, Carlo Dufour, Régis Peffault de Latour, Marc H. G. P. Raaijmakers, Neal Young

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
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

12:50-14:00 Lunch

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

15:30-15:40 Coffee break

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)
LIVE Programme
CET Time

SELECTED BRIEF ORAL COMMUNICATION

16:30-16:40  Natural History and Outcomes Depending on Organ System in 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)
LIVE Programme
CET Time

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 NOCTURNAL 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

6. Surveillance of leukaemia predisposition in bone marrow failure: How, and when to act?
Lucy Godley & Jaroslaw P. Maciejewski
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)
LIVE Programme
CET Time

16:35-17:05 Discussion

17:05-17:15 Coffee break

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