LIVE Programme
CET Time

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

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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)

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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)

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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

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Sunday, November 15, 2020

13h00-14:00 **SATELLITE SYMPOSIUM:**

Title: Current unmet need and the future of complement inhibition in PNH
Chair: Peter Hillmen and Antonio Risitano
Speakers: Peter Hillmen and Antonio Risitano

This symposium is organized by Apellis

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)*

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