

**2ND TRANSLATIONAL RESEARCH CONFERENCE
ERYTHROPOIESIS CONTROL AND INEFFECTIVE ERYTHROPOIESIS :
FROM BENCH TO BEDSIDE**

March 5-7, 2021

Chairpersons: Maria-Domenica Cappellini (Milan), Stuart Orkin (Boston), Valeria Santini (Florence)

Friday, March 5th, 2021

13:30-13:40 Welcome

SESSION I – Bone marrow response to viral infections

Chairs: Stuart Orkin (Boston), Valeria Santini (Florence)

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| 13:40-13:45 | Introduction | Stuart Orkin (Boston) Valeria Santini (Florence) |
| 13:45-14:05 | Immunity and variability response to infection | Jean-Laurent Casanova (New York) |
| 14:05-14:25 | Emergency hemopoiesis in viral infections | Jonas Schulte-Schrepping (Bonn) |
| 14:25-14:32 | <i>Enhanced HbF Reactivation By In Vivo Multiplex Mutagenesis of Thalassemic CD34+ Cells</i> | Aphrodite Georgakopoulou (Thessaloniki) |
| 14:32-14:39 | <i>Targeting TFR2: A Novel “Erythropoiesis-Stimulating” Approach</i> | Antonella Nai (Milan) |
| 14:39-15:10 | <i>Panel discussion</i> | |

15:10-16:15 Break

SESSION II – Haematological response to SARS CoV2 infection

Chair(s): Maria-Domenica Cappellini (Milan), Olivier Hermine (Paris)

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| 16:15-16:20 | Introduction | Maria-Domenica Cappellini (Milan) Olivier Hermine (Paris) |
| 16:20-16:35 | Iron and viral infection | Hal Drakesmith (Oxford) |
| 16:35-16:50 | Iron and Heme disruption in COVID-19 | Emanuela Tolosano (Turin) |
| 16:50-17:05 | Anaemias and COVID-19 | Irene Motta (Milan) |
| 17:05-17:20 | Focus on blood management during the COVID outbreak | Giancarlo M. Liumbruno (Rome) |
| 17:20-17:40 | Hematopoiesis in MDS and Covid 19 infection | Eric Solary (Paris) |
| 17:40-18:10 | <i>Panel discussion</i> | |

SESSION III – Dyserythropoiesis in clonal haemopoiesis and MDS

Chairs: Valeria Santini (Florence), Michaela Fontenay (Paris)

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| 18:10-18:15 | Introduction | Valeria Santini (Florence) Michael Fontenay (Paris) |
| 18:15-18:30 | Into the mechanisms of dyserythropoiesis : the SF3B1 mutation and RSP-14 deficiency models | Michaela Fontenay (Paris) |
| 18:30-18:45 | TGF pathway in ineffective erythropoiesis | Olivier Hermine (Paris) |
| 18:45-19:00 | Hypoxic niche and microenvironment in clonal haemopoiesis and MDS | Marc H.G.P. Raaijmakers (Rotterdam) |
| 19:00-19:07 | <i>Why is the translation selectivity of low ribosome availability deleterious for erythropoiesis?</i> | Ismael Boussaid (Paris) |
| 19:07-19:40 | <i>Panel discussion</i> | |

19:40-20:40 3 Simultaneous Meet the Expert Sessions

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| 1. Management of non-transfusion-dependent thalassaemias | Ali Taher (Beirut)/John Porter (London) |
| 2. New treatments in haemoglobinopathies | Antonio Piga (Turin) |
| 3. Anaemia and autoimmunity management in MDS. | Pierre Fenaux (Paris)/Arsene Mekinian (Paris) |

Saturday, March 6th, 2021

SESSION IV – Erythropoiesis Control

Chairs: Douglas Higgs (Oxford), Mark Fleming (Boston)

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| 13:30-13:35 | Introduction | Douglas Higgs (Oxford) Mark Fleming (Boston) |
| 13:35-13:50 | Congenital sideroblastic anaemias | Mark Fleming (Boston) |
| 13:50-14:05 | GATA 1 experience | Emery Bresnick (Madison) |
| 14:05-14:20 | Single cell analysis during erythropoiesis | Vijay G. Sankaran (Boston) |
| 14:20-14:27 | <i>Acquired α-Thalassemia (HbH disease) in a Patient with Myelodysplastic Syndrome with Excess Blasts - 1 (AT-MDS-EB-1) Reveals a Novel Splice-Donor Site Atrx variant on NGS</i> | Prashant Sharma (Chandigarh) |
| 14:27-15:00 | <i>Panel discussion</i> | |

SESSION V – Erythropoiesis control : phase 2

Chairs: Mohandas Narla (New York), Stuart Orkin (Boston)

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| 15:00-15:05 | Introduction | Mohandas Narla (New York) Stuart Orkin (Boston) |
| 15:05-15:20 | Erythroid progenitors | Lionel Blanc (New York) |
| 15:20-15:35 | Eryptosis | Mehrdad Ghashghaeinia (Tübingen) |

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| 15:35-15:50 | Bone marrow 3D modelling | Alessandra Balduini (Pavia) |
| 15:50-16:20 | <i>Panel discussion</i> | |
| 16:20-16:30 | <i>Break</i> | |

16:30-17:30 **SATELLITE SYMPOSIUM**

Clinical Scenarios in MDS and β -thalassemia: Expert Opinions on Managing Anemia
Chair: Aristoteles Giagounidis (Düsseldorf)
Speakers: María Díez Campelo (Salamanca), Antonis Kattamis (Athens)

This symposium is organized and sponsored by Bristol Myers Squibb.

SESSION VI – Iron metabolism and erythropoiesis

Chair: Clara Camaschella (Milan)

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| 17:30-17:35 | Introduction | Clara Camaschella (Milan) |
| 17:35-17:50 | Erythropoiesis and iron metabolism interaction | Laura Silvestri (Milan) |
| 17:50-18:05 | Macrophage and iron | Martina Muckenthaler (Heidelberg) |
| 18:05-18:20 | Atypical microcytic anaemias | Graça Porto (Porto) |
| 18:20-18:27 | <i>Ker-050, a Novel Inhibitor of Tgfb Superfamily Signaling, Induces Red Blood Cell Production By Promoting Multiple Stages of Erythroid Differentiation</i> | Marina Feigenson (Lexington) |
| 18:27-19:00 | <i>Panel discussion</i> | |

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

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| 1. Regulation of globin gene switches: embryonic to fetal and fetal to adult. | Douglas Higgs (Oxford)/Stuart Orkin (Boston) |
| 2. Mechanisms of inflammation, innate and adaptive Immune responses in MDS | Shahram Kordasti (London)/Jonathan Irish (Nashville) |

19:00-20:00 Mentored Poster Walk

Leaders for Biology: *Michaela Fontenay (Paris) & Tomas Ganz (Los Angeles)*
Leaders for Clinical: *Maria Díez-Campelo (Salamanca) & Maria-Domenica Cappellini (Milan)*

Sunday, March 7th, 2021

SESSION VII – Inherited dyserythropoiesis

Chair: Achille Iolascon (Naples)

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| 14:00-14:05 | Introduction | Achille Iolascon (Naples) |
| 14:05-14:20 | Congenital dyserythropoietic anaemia | Roberta Russo (Naples) |

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| 14:20-14:35 | Diamond-Blackfan anaemia | Lydie Da Costa (Paris) |
| 14:35-14:50 | Germline predisposition to MDS and bone marrow failure | Akiko Shimamura (Boston) |
| 14:50-14:57 | <i>Haploidentical Stem Cell Transplantation for High Risk Haemoglobinopathies Using Post-Transplant Cyclophosphamide</i> | Du' A Darabseh (Amman) |
| 14:57-15:30 | <i>Panel discussion</i> | |

SESSION VIII – Gene Therapy/Editing

Chairs: John Porter (London), Maria-Domenica Cappellini (Milan)

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| 15:30-15:35 | Introduction | John Porter (London) Maria-Domenica Cappellini (Milan) |
| 15:35-15:50 | Who is the best patient candidate for gene therapy? | Vip Viprakasit (Bangkok) |
| 15:50-16:05 | Gene therapy in haemoglobinopathies vs BMT | Marina Cavazzana (Paris) |
| 16:05-16:20 | Gene therapy in haemoglobinopathies vs gene editing | Mitchell Weiss (Memphis) |
| 16:20-16:35 | Focus on : Immunotherapy and CAR-T in MDS: Dream or reality? | Rafael Bejar (La Jolla) |
| 16:35-17:05 | <i>Panel discussion</i> | |

17:05-17:15 Break

SESSION IX – Drugs and ineffective erythropoiesis

Chairs: Ali Taher (Beirut), Antonio Piga (Turin)

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| 17:15-17:20 | Introduction | Ali Taher (Beirut) Antonio Piga (Turin) |
| 17:20-17:35 | Iron restricted erythropoiesis | Tomas Ganz (Los Angeles) |
| 17:35-17:50 | New approaches to anaemia of MDS | Uwe Platzbecker (Leipzig) |
| 17:50-18:05 | New approaches to the management of sickle cell disease | Lucia De Franceschi (Verona) |
| 18:05-18:12 | <i>Inclusion of a shRNA Targeting BCL11A into a β-Globin Expressing Vector Allows Concurrent Synthesis of Curative Adult and Fetal Hemoglobin</i> | Danuta Jarocho (Philadelphia) |
| 18:12-18:42 | <i>Panel discussion</i> | |
| 18:42-19:00 | Closing remarks | |