

**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)

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)

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)

18:10-18:15	Introduction	Valeria Santini (Florence) Michaela 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)

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)

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)

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)

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	2 Simultaneous Meet the Expert sessions	
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)

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

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)

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	<i>Break</i>	

SESSION IX – Drugs and ineffective erythropoiesis

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

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	