3rd Translational Research Conference
Erythropoiesis Control and Ineffective Erythropoiesis:
from bench to bedside
March 3-5, 2023 - Paris, France
Chairpersons : Maria-Domenica Cappellini, Michaela Fontenay, Stuart Orkin

Friday, March 3rd, 2023

08:30–08:40 Welcome

SESSION I – Metabolism in erythroid cells
Chairs: Stuart Orkin (Boston), Michaela Fontenay (Paris)

08:40-08:45	Introduction	Stuart Orkin (Boston) Michaela Fontenay (Paris)
08:45-09:05	A glutamine metabolic switch regulates normal and ineffective erythropoiesis	Jian Xu (Texas)
09:05- 09:15	*Discussion*	
09:15-09:35	Arginine metabolism regulates human erythroid differentiation through hypusination of eIF5 A	Sandrina Kinet (Montpellier)
09:35-09:45	*Discussion*	
09:45-10:05	TIF1γ regulates nucleotide and mitochondrial metabolism, and counteracts ferroptosis to drive erythroid progenitor differentiation	Marlies Rossmann (Rochester)
10:05-10:15	*Discussion*	
10:15-10:22	**Selected brief oral communication:** The role of hypoxia and inflammation in the regulation of iron metabolism and erythropoiesis in COVID-19: the IRONCOVID Study	Diletta Maira (Milan)
10:22-10:25	*Discussion*	
10:25-10:45	*Panel discussion*	

10:45-11:15 Coffee Break

SESSION II –Proteome remodeling and cellular clearance in erythropoiesis
Chairs: Maria-Domenica Cappellini (Milan), Mohandas Narla (New York)

11:15-11:20	Introduction	Maria-Domenica Cappellini (Milan) Mohandas Narla (New York)

11:20-11:40	M6A epitranscriptome-mediated RNA stress granule assembly governs red blood cell development	Leonard I. Zon (Boston)
11:40-11:50	*Discussion*	
11:50-12:10	Global Proteome Remodeling in Erythroid Differentiation	Daniel Finley (Boston)
12:10-12:20	*Discussion*	
12:20-12:40	Erythroblast Enucleation and Reticulocyte Biology	Mohandas Narla (New York)
12:40-12:50	*Discussion*	
12:50-12:57	**Selected brief oral communication:** Macrophage type I IFN sensing facilitates stress erythropoiesis	Florian Deckert (Vienna)
12:57-13:00	*Discussion*	
13:00-13:20	*Panel discussion*	
13:20-15:00	***Lunch***	

SESSION III – Fetal globin silencing
Chairs: Douglas Higgs (Oxford), Scott Peslak (Philadelphia)

15:00-15:05	Introduction	Douglas Higgs (Oxford) Scott Peslak (Philadelphia)
15:05-15:25	Cellular signaling pathways in the regulation of fetal hemoglobin for treatment of sickle cell disease	Scott Peslak (Philadelphia)
15:25-15:35	*Discussion*	
15:35-15:55	the Role of Sox6 and Coup-TFII in controlling gamma-globin transcription	Antonella Ronchi (Milan)
15:55-16:05	*Discussion*	
16:05-16:25	Reversal of hemoglobin switching by cis-regulatory interference	Jennifer Cherone (Seattle)
16:25-16:35	*Discussion*	
16:35-16:42	**Selected brief oral communication:** Novel lentiviral vectors for gene therapy of sickle cell disease combining gene addition and gene silencing strategies	Megane Brusson (Paris)
16:42-16:45	*Discussion*	
16:45-17:05	*Panel discussion*	
17:05-17:35	***Coffee break***	

SESSION IV – Bone marrow cell expansion and engraftment
Chairs: Giuliana Ferrari (Milan), Stefano Rivella (Philadelphia)

17:35-17:40	Introduction	Giuliana Ferrari (Milan) Stefano Rivella (Philadelphia)

17:40-18:00	Bone marrow transplantation in haemoglobinopathies	Franco Locatelli (Rome)
18:00-18:10	*Discussion*	
18:10-18:30	Bone marrow transplantation in high-risk MDS	Raynier Devillier (Marseille)
18:30-18:40	*Discussion*	
18:40-19:00	Gene therapy for beta-thalassemia: what do we know about hematopoiesis in Bthal?	Giuliana Ferrari (Milan)
19:00-19:10	*Discussion*	
19:10-19:17	**Selected brief oral communication:** Novel, potent, compact, erythroid specific enhancers successfully replace the beta globin-uLCR in gene therapy vectors for beta hemoglobinopathies	Kiriaki Paschoudi (Thessaloniki)
19:17-19:20	*Discussion*	
19:20-19:40	*Panel discussion*	

19:40 – 20:40 Mentored Poster Walks and Welcome Get Together stand up buffet dinner

Clinical poster leaders : Achille Iolascon (Naples) & Mitchell J. Weiss (Memphis)
Biology poster leaders : Leonard I. Zon (Boston) & Antonella Ronchi (Milan)

Saturday, March 4th, 2023

09:00-10:00	**3 Simultaneous Meet the Expert sessions (non-clinical)**	
	1, Gene therapy for thalassemia	Giuliana Ferrari (Milan)
	2, Gene regulation	Douglas Higgs (Oxford)
	3, Fetal haemoglobin switching	Stuart Orkin (Boston)
10:00-10:30	***Coffee break***	

SESSION V – Stressed erythropoiesis in MDS
Chairs: Michaela Fontenay (Paris), Cristian Bellodi (Lund)

10:30-10:35	Introduction	Michaela Fontenay (Paris) Cristian Bellodi (Lund)
10:35-10:55	R-loops and genome instability phenotypes in splicing-deficient conditions	Frédéric Chédin (Davis)
10:55-11:05	*Discussion*	
11:05-11:25	Transcriptional stress by RNA splicing alterations in MDS	Salima Benbarche (New York)
11:25-11:35	*Discussion*	
11:35-11:55	Alterations of translation control by RNA modification in MDS	Cristian Bellodi (Lund)
11:55-12:05	*Discussion*	
12:05-12:12	**Selected brief oral communication:** Ineffective Erythropoiesis of Myelodysplastic Syndromes with SF3B1 Mutation Involves a DNA Replication Stress Due to Loss of R-Loopss	David Rombaut (Paris)
12:12-12:15	*Discussion*	

12:15-12:35 *Panel discussion*

12:35-14:15 ***Lunch***

13:05-14:05 **SATELLITE SYMPOSIUM:**
Managing Ineffective Erythropoiesis and Its Importance in Non-Transfusion-Dependent β-thalassemia
Chair: Olivier Hermine (Paris)
Speakers: Stefano Rivella (Philadelphia), Ali Taher (Lebanon)

This educational symposium is organized and funded by Bristol Myers Squibb

SESSION VI – Stressed erythropoiesis in haemoglobinopathies and other haemolytic anaemia
Chairs: Achille Iolascon (Naples), Mitchell J. Weiss (Memphis)

14:15-14:20	Introduction	Achille Iolascon (Naples) Mitchell J. Weiss (Memphis)
14:20-14:40	PIEZO1 in xerocytosis/dehydrated hereditary stomatocytosis	Immacolata Andolfo (Naples)
14:40-14:50	*Discussion*	
14:50-15:10	Regulation of normal and diseased erythropoiesis by transferrin	Stefano Rivella (Philadelphia)
15:10-15:20	*Discussion*	
15:20-15:40	P. falciparum infection of erythroid cells	Catherine Lavazec (Paris)
15:40-15:50	*Discussion*	
15:50-15:57	**Selected brief oral communication:** GATA1 regulates RPS19 and RPL11 Ribosomal Protein genes in erythroid cells	Cecilia Ng (London)
15:57-16:00	*Discussion*	

16:00-16:20 *Panel discussion*

16:20-16:50 ***Coffee break***

SESSION VII – Clinical and diagnosis
Chair: Maria-Domenica Cappellini (Milan)

16:50-16:55	Introduction	Maria-Domenica Cappellini (Milan)
16:55-17:15	How to do the molecular diagnosis for rare anaemias	Noemi Roy (Oxford)
17:15-17:25	*Discussion*	
17:25-17:45	Methemoglobinaemia	Wilma Barcellini (Milan) *virtual*
17:45-18:05	Congenital dyserythropoietic anemia	Achille Iolascon (Naples)
18:05-18:15	*Discussion*	

18:15-18:22	**Selected brief oral communication:** Modeling of congenital dyserythropoietic anemia type II for developing lentiviral-mediated hematopoietic stem cell gene therapy	Oscar Quintana Bustamante (Madrid)
18:22-18:25	*Discussion*	
18:25-18:55	*Panel discussion*	

Sunday, March 5th, 2023

07:30-08:30 3 Simultaneous Breakfast Meet the Expert Sessions (Clinical)

1, Zebrafish and hematopoiesis	Leonard I. Zon (Boston)
2, R-loops in vivo	Frédéric Chédin (Davis)
3, Treatment of ineffective erythropoiesis	Olivier Hermine (Paris)

SESSION VIII – Gene therapy and genome editing
Chairs: Daniel Bauer (Boston), Giuliana Ferrari (Milan)

08:35-08:40	Introduction	Daniel Bauer (Boston) Giuliana Ferrari (Milan)
08:40-09:00	Hematopoietic stem cell gene editing	Daniel Bauer (Boston)
09:00-09:10	*Discussion*	
09:10-09:30	CRISPR/Vertex SCD and Thal trial	Selim Corbacioglu (Regensburg)
09:30-09:40	*Discussion*	
09:40-10:00	Base editing for hemoglobin disorders	Mitchell J. Weiss (Memphis)
10:00-10:10	*Discussion*	
10:10-10:17	**Selected brief oral communication:** Prime editing to correct sickle cell disease mutation results in high editing rates and improves the disease phenotype	Kiriaki Paschoudi (Thessaloniki)
10:17-10:20	*Discussion*	
10:20-10:40	*Panel discussion*	
10:40-11:10	***Coffee break***	

SESSION IX – Pharmacological approaches
Chairs: Maria-Domenica Cappellini (Milan), Martina Muckenthaler (Heidelberg)

11:10-11:15	Introduction	Maria-Domenica Cappellini (Milan) Martina Muckenthaler (Heidelberg)
11:15-11:35	Ineffective erythropoiesis treatment	Olivier Hermine (Paris)
11:35-11:45	*Discussion*	
11:45-12:05	Iron Metabolism Control	Martina Muckenthaler (Heidelberg)
12:05-12:15	*Discussion*	
12:15-12:35	RBC Metabolism manipulation	Kevin Kuo (Toronto)
12:35-12:45	*Discussion*	

12:45-12:52 **Selected brief oral communication:**
Genetic iron overload exacerbates and pharmacological iron restriction improves MDS pathophysiology in preclinical studies — Francesca Vinchi (New York)

12:52-12:55 *Discussion*

12:55-13:15 Panel discussion

13:15-13:25 Closing Remarks

MEETING CLOSURE