



**3<sup>rd</sup> 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 3<sup>rd</sup>, 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	Glutamine switch	Jian Xu (Texas)
09:05- 09:15	<i>Discussion</i>	
09:15-09:35	Arginine metabolism regulates human erythroid differentiation through hypusination of eIF5 A	Sandrina Kinet (Montpellier)
09:35-09:45	<i>Discussion</i>	
09:45-10:05	Coenzyme Q ferroptosis TIF1g	Marlies Rossmann (Rochester)
10:05-10:15	<i>Discussion</i>	
10:15-10:22	<b>Selected brief oral communication:</b> 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	<i>Discussion</i>	
10:25-10:45	<i>Panel discussion</i>	
10:45-11:15	<b>Coffee Break</b>	

**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)
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Draft Programme January 30, 2023

11:20-11:40	M6A epitranscriptome-mediated RNA stress granule assembly governs red blood cell development	Leonard I. Zon (Boston)
11:40-11:50	<i>Discussion</i>	
11:50-12:10	Global Proteome Remodeling in Erythroid Differentiation	Daniel Finley (Boston)
12:10-12:20	<i>Discussion</i>	
12:20-12:40	Erythroblast Enucleation and Reticulocyte Biology	Mohandas Narla (New York)
12:40-12:50	<i>Discussion</i>	
12:50-12:57	<b>Selected brief oral communication:</b> Macrophage type I IFN sensing facilitates stress erythropoiesis	Florian Deckert (Vienna)
12:57-13:00	<i>Discussion</i>	
13:00-13:20	<i>Panel discussion</i>	
13:20-15:00	<b>Lunch</b>	
13:50-14:50	<b>Possible slot for a satellite symposium</b>	

### 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	<i>Discussion</i>	
15:35-15:55	the Role of Sox6 and Coup-TFII in controlling gamma-globin transcription	Antonella Ronchi (Milan)
15:55-16:05	<i>Discussion</i>	
16:05-16:25	Cis-regulatory interference	Jennifer Cherone (Seattle)
16:25-16:35	<i>Discussion</i>	
16:35-16:42	<b>Selected brief oral communication:</b> Novel lentiviral vectors for gene therapy of sickle cell disease combining gene addition and gene silencing strategies	Megane Brusson (Paris)
16:42-16:45	<i>Discussion</i>	
16:45-17:05	<i>Panel discussion</i>	
17:05-17:35	<b>Coffee break</b>	

### SESSION IV – Bone marrow cell expansion and engraftment

**Chair(s): TBA**

Draft Programme January 30, 2023

17:35-17:40	Introduction	Session Chair
17:40-18:00	Bone marrow transplantation in haemoglobinopathies	Franco Locatelli (Rome)
18:00-18:10	<i>Discussion</i>	
18:10-18:30	Bone marrow transplantation in high-risk MDS	Raynier Devillier (Marseille)
18:30-18:40	<i>Discussion</i>	
18:40-19:00	Gene therapy for beta-thalassemia: what do we know about hematopoiesis in Bthal?	Giuliana Ferrari (Milan)
19:00-19:10	<i>Discussion</i>	
19:10-19:17	<b>Selected brief oral communication:</b> 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	<i>Discussion</i>	
19:20-19:40	<i>Panel discussion</i>	
19:40 – 20:40	<b>Mentored Poster Walks and Welcome Get Together stand up buffet dinner</b>	

## Saturday, March 4<sup>th</sup>, 2023

08:00-09:00	<b>3 Simultaneous Meet the Expert sessions (non-clinical)</b> R-loops in vivo Gene regulation Fetal haemoglobin switching	Frédéric Chédin (California) Douglas Higgs (Oxford) Stuart Orkin (Boston)
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**09:00-10:00 Possible slot for a satellite symposium**

**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 (California)
10:55-11:05	<i>Discussion</i>	
11:05-11:25	Transcriptional stress by RNA splicing alterations in MDS	Salima Benbarche (New York)
11:25-11:35	<i>Discussion</i>	
11:35-11:55	Alterations of translation control by RNA modification in MDS	Cristian Bellodi (Lund)
11:55-12:05	<i>Discussion</i>	
12:05-12:12	<b>Selected brief oral communication:</b> Ineffective Erythropoiesis of Myelodysplastic Syndromes with SF3B1 Mutation Involves a DNA Replication Stress Due to Loss of R-Loopss	David Rombaut (Paris)

Draft Programme January 30, 2023

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  $\beta$ -thalassaemia**

**Chairs:** Olivier Hermine (Paris)

**Speakers:** Stefano Rivella (Philadelphia)

*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	<i>Discussion</i>	
14:50-15:10	Regulation of normal and diseased erythropoiesis by transferrin	Stefano Rivella (Philadelphia)
15:10-15:20	<i>Discussion</i>	
15:20-15:40	<i>P. falciparum</i> infection of erythroid cells	Catherine Lavazec (Paris)
15:40-15:50	<i>Discussion</i>	
15:50-15:57	<b>Selected brief oral communication:</b> GATA1 regulates RPS19 and RPL11 Ribosomal Protein genes in erythroid cells	Cecilia Ng (London)
15:57-16:00	<i>Discussion</i>	
16:00-16:20	<i>Panel discussion</i>	
16:20-16:50	<b><i>Coffee break</i></b>	

**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	<i>Discussion</i>	
17:25-17:45	Methemoglobinaemia	Wilma Barcellini (Milan)

Draft Programme January 30, 2023

17:45-17:55 *Discussion*

17:55-18:15 Congenital dyserythropoietic anemia

Achille Iolascon (Naples)

18:15-18:25 *Discussion*

18:25-18:32 **Selected brief oral communication:**

Modeling of congenital dyserythropoietic anemia type II  
for developing lentiviral-mediated hematopoietic stem cell gene therapy

Oscar Quintana Bustamante (Madrid)

18:32-18:35 *Discussion*

18:35-18:55 *Panel discussion*

**19:05-20:05 Possible slot for a satellite symposium**

## Sunday, March 5<sup>th</sup>, 2023

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

Zebrafish and hematopoiesis

Leonard I. Zon (Boston)

Gene therapy for thalassemia

Franco Locatelli (Rome)

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)

Draft Programme January 30, 2023

11:15-11:35	Ineffective erythropoiesis treatment	Olivier Hermine (Paris)
11:35-11:45	<i>Discussion</i>	
11:45-12:05	Iron Metabolism Control	Martina Muckenthaler (Heidelberg)
12:05-12:15	<i>Discussion</i>	
12:15-12:35	RBC Metabolism manipulation	Kevin Kuo (Toronto)
12:35-12:45	<i>Discussion</i>	
12:45-12:52	<b>Selected brief oral communication:</b> Genetic iron overload exacerbates and pharmacological iron restriction improves MDS pathophysiology in preclinical studies	Francesca Vinchi (New York)
12:52-12:55	<i>Discussion</i>	
12:55-13:15	<i>Panel discussion</i>	
13:15-13:25	<b><i>Closing Remarks</i></b>	

**MEETING CLOSURE**