International conference
ERYTHROPOIESIS CONTROL AND INEFFECTIVE ERYTHROPOIESIS: FROM BENCH TO BEDSIDE

Budapest, Hungary
March 15-17, 2019
Chairs: Maria Domenica Cappellini (Milan), Stuart Orkin (Boston), Valeria Santini (Florence)

Friday, March 15, 2019

08:30–08:45 Welcome

SESSION I – ERYTHROPOIESIS CONTROL 1
Chair: Douglas Higgs

08:45-09:00	Origin of mega/erythroid progenitors in haematopoiesis	Claus Nerlov (Oxford)
09:00-09:05	*Discussion*	
09:05-09:20	Transcription factors in erythropoiesis	John Strouboulis (London)
09:20-09:25	*Discussion*	
09:25-09:40	Mitochondria coordinate metabolism and epigenetic gene regulation during normal and ineffective erythropoiesis	Jian Xu (Dallas)
09:40-09:45	*Discussion*	
09:45-10:00	The biochemical programme of extreme terminal differentiation	Daniel Finley (Boston)
10:00-10:05	*Discussion*	
	Brief oral communication	
10:05-10:10	Erythroid differentiation is regulated by stage-specific changes in the TCA cycle	Pedro Gonzalez-Menendez (Montpellier)
10:10-10:13	*Discussion*	
10:13-10:35	*Panel discussion*	

10:35-11:05 Coffee Break

SESSION II – INEFFECTIVE ERYTHROPOIESIS
Chairs: Mohandas Narla, Achille Iolascon

11:05-11:20	Markers of ineffective erythropoiesis	John Porter (London)
11:20-11:25	*Discussion*	
11:25-11:40	Congenital dyserythropoietic anaemias (CDAs)	Achille Iolascon (Naples)
11:40-11:45	*Discussion*	
11:45-12:00	Ineffective erythropoiesis in thalassaemia syndromes	Stefano Rivella (Philadelphia)

12:00-12:05 *Discussion*

12.05-12.20 Ineffective erythropoiesis in myelodysplastic syndromes — Michaela Fontenay (Paris)
12.20-12.25 *Discussion*

Brief oral communications

12:25-12:30 A variant erythroferrone induced by clonal erythropoiesis disrupts iron homeostasis in SF3B1-mutated myelodysplastic syndrome — Sabrina Bondu (Paris)
12:30-12:33 *Discussion*

12:33-12:38 Faithful recapitulation of human acute erythroid Lleukaemia by multiplex CRISPR/Cas9-based genome editing of mouse haematopoietic stem cells — Ilaria Iacobucci (Memphis)
12:38-12:41 *Discussion*

12:41-12:55 *Panel discussion*

13:00-14:00 Lunch

SESSION III - ERYTHROPOIESIS CONTROL 2
Chair: Stefano Rivella

14:00-14:20 Single cell RNA-SEQ analysis of the haematopoietic and the erythroid trajectories — Merav Socolovsky (Worcester)
14:20-14:25 *Discussion*

14:25-14:45 IPS modeling of red cell disorders, including DBA — Sergei Doulatov (Seattle)
14:45-14:50 *Discussion*

Brief oral communication

14:50-14:55 Inactivation of the nuclear interacting SET domain protein 1 (NSD1) impairs terminal erythroid maturation and results in erythroleukemia in mice — Samantha Tauchmann (Basel)
14:55-14:58 *Discussion*

14:58-15:30 *Panel discussion*

15:30-16:00 Coffee break

SESSION IV - CLINICAL CONSEQUENCES OF INEFFECTIVE ERYTHROPOIESIS: CASE PRESENTATIONS
Chair: Lucia de Franceschi

16:00-16:15 Congenital dyserythropoietic anaemias — Roberta Russo (Naples)
16:15-16:20 *Discussion*

16:20-16:35 Managing medical complications of beta-thalassaemia in adults — Ali Taher (Beirut)
16:35-16:40 *Discussion*

16:40-16:55 Clinical consequences of ineffective erythropoiesis — Aristoteles Giagounidis (Düsseldorf)

	in myelodysplastic syndromes	
16:55-17:00	*Discussion*	
17:00-17:15	Hereditary haemolytic anaemias	Immacolata Andolfo (Naples)
17:15-17:20	*Discussion*	
17:20-17:35	Sickle cell disease	Mariane de Montalembert (Paris)
17:35-17:40	*Discussion*	
17:40-17:55	Acute sickling crisis	Lucia De Franceschi (Verona)
17:55-18:00	*Discussion*	
	Brief oral communication	
18:00-18:05	Genomic-DNA exposed by somatic gene mutations engages the Cgas/Sting axis to license the NLRP3 inflammasome in myelodysplastic syndromes (MDS)	Amy McLemore (Tampa)
18:05-18:08	*Discussion*	
18:08-18:30	*Panel discussion*	

18:30 ***Poster Walks and Get-Together Welcome Reception***

- Biology poster walk : Sergei Doulatov (Seattle), Maria-Domenica Cappellini (Milan)
- Clinical poster walk : Wilma Barcellini (Milan), Aristoteles Giagounidis (Düsseldorf)

Saturday, March 16, 2019

09:00-10:00 3 Simultaneous Meet the Expert Sessions (non-clinical)

1.	Study of Normal and Disordered Human Erythropoiesis	Mohandas Narla (New York)
2.	Control of Hb switching	Douglas Higgs (Oxford)
3.	Heme regulation	Janis Abkowitz (Seattle)

10:00-10:25 Coffee break

SESSION V – HAEMOGLOBIN SWITCHING
Chairs: John Porter, Maria-Domenica Cappellini

10:25-10:40	The relationship between genome structure and function : lessons from the globin loci	Douglas Higgs (Oxford)
10:40-10:45	*Discussion*	
10:45-11:00	Promoter mutations associated with hereditary persistence of fetal haemoglobin either disrupt repressors or create de novo sites for activators	Merlin Crossley (Sydney)
11:00-11:05	*Discussion*	
11:05-11:20	Exploiting targeted (Epi)Genome Editing for therapeutic applications	Angelo Lombardo (Milan)
11:20-11:25	*Discussion*	
11:25-11:40	Haemoglobin switching: reactivation	Stuart Orkin (Boston)
11:40-11:45	*Discussion*	

11:45-12:15 *Panel discussion*

12:15-12:25 **ESH International Scholarship Fund** — Maria-Domenica Cappellini (Milan)
Early-Career Scholarship Award Ceremony — Stuart Orkin (Boston)
Valeria Santini (Florence)

12:25-14:05 Lunch

12:50-13:50 **SATELLITE SYMPOSIUM:**
Clinical success treating ineffective erythropoiesis
Chair: Maria-Domenica Cappelini (Milan)
Speakers: Olivier Hermine (Paris), Alan List (Tampa)

This symposium is organised and funded by Celgene.

SESSION VI – EMERGENCY IN RED CELL DISORDERS
Chairs: Vip Viprakasit, Valeria Santini

14:05-14:25 Fanconi anaemia — Carlo Dufour (Genoa)
14:25-14:30 *Discussion*

14:30-14:50 Autoimmune haemolytic anaemias — Wilma Barcellini (Milan)
14:50-14:55 *Discussion*

14:55-15:15 Emergency in thalassaemia — Ali Taher (Beirut)
15:15-15:20 *Discussion*

Brief oral communication

15:20 -15:25 Erythroid niche formation in the bone marrow is dependent on constant recruitment of monocytes for efficient erythroid output — Verena Petzer (Innsbruck)
15:25 -15:28 *Discussion*

15:28-15:55 *Panel discussion*

15:55-16:20 Coffee Break

SESSION VII – PHARMACOLOGICAL TREATMENT OF INEFFECTIVE ERYTHROPOIESIS
Chair: Ali Taher

16:20-16:35 Inflammation as a determinant of ineffective — Alan List (Tampa)

	erythropoiesis in myelodysplastic syndromes	
16:35-16:40	*Discussion*	
16:40-16:55	TGF-β pathway inhibitors in thalassaemias	Vip Viprakasit (Bangkok)
16:55-17:00	*Discussion*	
17:00-17:15	TGF-β pathway inhibitors in low risk MDS	Valeria Santini (Florence)
17:15-17:20	*Discussion*	
17:20-17:35	Iron restricted erythropoiesis	John Porter (London)
17:35-17:40	*Discussion*	
17:40-17:55	New approches to managing sickle cell disease	David Rees (London)
17:55-18:00	*Discussion*	
18:00-18:15	Heme regulation and GATA1	Janis Abkowitz (Seattle)
18:15-18:20	*Discussion.*	
	Brief oral communication	
18:20-18:25	CXXC5 (RINF) loss-of-expression accelerates human erythroid differentiation and attenuates red cell expansion in a TGFb-and SMAD7-dependent manner	Frederic Pendino (Paris)
18:25-18:28	*Discussion*	
18:28-18:50	*Panel discussion*	

Sunday, March 17, 2019

07:40-8:40 3 Simultaneous Meet the Expert Sessions (Clinical)

1. Role of TGF-beta superfamily in ineffective erythropoiesis	Amit K. Verma (New-York)
2. Pre-clinical part of abnormal erythropoiesis	Michaela Fontenay (Paris)
3. Iron restricted erythropoiesis : what does it mean ?	John Porter (London)

SESSION VIII – GENE THERAPY/EDITING
Chair: Angelo Lombardo

08:45-09:00	Gene modification : history and principles	Fyodor Urnov (Seattle)
09:00-09:05	*Discussion*	
09:05-09:20	Gene editing for thalassemia and sickle cell disease	Daniel Bauer (Boston)
09:20-09:25	*Discussion*	
09:25-09:40	Gene therapy of thalassaemia / sickle cell disease	Giuliana Ferrari (Milan)
09:40-09:45	*Discussion*	
09:45-10:00	Flipping the switch : initial results of genetic targeting of the fetal to adult globin switch in sickle cell patients	Christian Brendel (Boston)
10:00-10:05	*Discussion*	

10:05-10:20	Gene therapy for thalassaemia / sickle cell disease	Alexis A. Thompson (Chicago)
10:20-10:25	*Discussion*	

Brief oral communication

10:25-10:30	Novel chromatin insulators for increased efficacy of hematopoietic stem cell gene therapy	Penelope Georgia Papayanni (Thessaloniki)
10:30-10:33	*Discussion*	

10:33-10:55 *Panel discussion*

10:55-11:25 Coffee Break

SESSION IX – LATE BREAKING SESSION
Chairs: Valeria Santini, Maria-Domenica Cappellini

11:25-11:40	Pro-atherogenic and pro-inflammatory role of iron : relevance for chronically transfused patients	Francesca Vinchi (New-York)
11:40-11:45	*Discussion*	
11:45-12:00	Diamond-Blackfan anaemia : genetic and clinical complexity exemplified by 3 cases encountered in clinical practice	Marcin Wlodarski (Memphis)
12:00-12:05	*Discussion*	

Brief oral communication

12:05-12:10	The Serine/Threonine Kinase Mrckα, a Novel Partner of Transferrin Receptor Type 2, Is Involved in Erythropoiesis Regulation.	Cyrielle Richard (Paris)
12:10-12:13	*Discussion*	

12:13-12:45 *Panel discussion*

12:45-13:00 Closing Remarks

MEETING CLOSURE