



## CONFERENCE/WORKSHOP

# Innovative Therapies for Red Cell and Iron related Disorders

*April 15-18, 2010 / Cascais, Portugal*

### Organizing Committee:

*Clara Camaschella, Eliane Gluckman, Philippe Leboulch, Carole Beaumont, Mohandas Narla, Yves Beuzard*

### Invited speakers:

Christopher Baum (Hannover), Carole Beaumont (Paris), Françoise Bernaudin (Creteil), Yves Beuzard (Fontenay), Clara Camaschella (Milano), Francois-Loic Cosset (Lyon), Ioav Cabantchick (Jerusalem), Ivana de Domenico (Salt Lake City), Frederic Galacteros (Creteil), Eliane Gluckman (Paris), Frank Grosveld (Rotterdam), Keith Humphries (Vancouver), Philippe Leboulch (Fontenay/Boston), Franco Locatelli (Pavia), Narla Mohandas (New-York), Elizabeta Nemeth (Los Angeles), Stuart Orkin (Boston), Marie-Paul Roth (Toulouse), Miguel Soares (Lisbon), Pierre-Louis Tharaux (Paris), Swe Lee Thein (London)

### Objective :

- 1) Stimulation of research on innovative therapies of red cell and iron disorders, from targets to bed-side, including preclinical and clinical trials.
- 2) Presentation of original results (invited speakers, selected oral presentations and posters).
- 2) Cooperation between clinicians and scientists, in order to define new targets, to develop and evaluate experimental therapies.

# Program

## Thursday, April 15<sup>th</sup> 2010

### 20h00-21h00: KEYNOTE LECTURE I

The therapeutic potential of iPS cells

pending

## Friday, April 16<sup>th</sup> 2010

### 8h30-10h30: SESSION I: HEMATOPOIETIC STEM CELL (HSC) THERAPIES

*Chair: E. Gluckman (Paris)*

Allogeneic BMT in Sickle cell anemia  
BMT versus CB-HSC in hemoglobinopathies  
New Trends in HSC therapies  
Panel Discussion

F. Bernaudin (Creteil)  
E. Gluckman (Paris)  
F. Locatelli (Pavia)

*Selected oral presentations (15 min each)*

**10h30-11h00: Coffee break**

### 11h00-13h00: SESSION II: FROM HSC TO ERYTHROCYTE

*Chair: F. Grosveld (Rotterdam)*

Expansion of HSCs  
The molecular switching in erythroid differentiation  
Programming the hemoglobin genes for therapies  
Overcoming insertional adverse events of  
integrating gene vectors

K. Humphries (Vancouver)  
S. Orkin (Boston)  
F. Grosveld (Rotterdam)  
C. Baum (Hannover)

**13h00-15h00: Lunch**

### 15h00-17h30: SESSION III: GENE THERAPY

*Chair: P. Leboulch (Fontenay/Boston)*

*Selected oral presentations (15 min each)*

Gene therapy of hemoglobin disorders  
Design of lentiviral Vectors

P. Leboulch (Fontenay/Boston)  
F-L. Cosset (Lyon)

*Selected oral presentations (15 min each)*

**17h30-18h00: Coffee break**

### 18h00-19h00: POSTER SESSION I: BIOTHERAPIES

## Saturday, April 17<sup>th</sup> 2010

### 8h30-10h30: SESSION IV: IRON REGULATORY PATHWAY FOR THERAPIES

*Chairs: C. Camaschella (Milano)*

Heme and Iron recycling  
Role of Hemojuvelin in iron deficiency and overload  
The essential role of BMP6 in hepcidin regulation

C. Beaumont (Paris)  
C. Camaschella (Milano)  
M.P. Roth (Toulouse)

*Selected oral presentations (15 min each)*

**10h30-11h00: Coffee break**

**11h00-13h00: SESSION V: THE HEPCIDIN EFFECTOR PATHWAY FOR THERAPIES**

**Chair: C. Beaumont (Paris)**

Hepcidin agonists and antagonists	E. Nemeth (Los Angeles)
Targeting the hepcidin binding site of ferroportin	I. De Domenico (Salt Lake City)
Relocating misdistributed iron by chelators	I. Cabantchick (Jerusalem)

*Selected oral presentations (15 min each)*

**13h00-15h00: Lunch**

**15h00-17h30: SESSION VI: INNOVATIVE THERAPIES FOR RED CELL DISORDERS (I)**

**Chair: M. Narla (New-York)**

Erythrocyte acting therapies	Y. Beuzard (Fontenay)
Gene active therapies	S.L. Thein (London)
EPO therapy in hemoglobin disorders	F. Galacteros (Creteil)

*Selected oral presentations (15 min each)*

**17h30-18h00: Coffee break**

**18h00-19h00: POSTER SESSION II: IRON AND ERYTHROCYTE TARGETING THERAPIES**

**Sunday, April 18<sup>th</sup> 2010**

**9h00-10h00: SESSION VII: INNOVATIVE THERAPIES FOR RED CELL DISORDERS (II)**

**Chair: F. Galacteros (Creteil)**

Vascular acting therapies in Sickle cell disease	P-L. Tharaux (Paris)
Heme oxygenase protects against red cell disorders	M. Soares (Lisbon)

**10h00-10h30: Coffee break**

**10h30-11h30: KEYNOTE LECTURE II**

Erythrocyte targeting for therapies in malaria	M. Narla (New-York)
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***End of the meeting***