In collaboration with

Fondazione CARIPARO

Associazione Veneta per la Lotta contro la Talassemia

ALT - Associazione Lotta alla Talassemia di Ferrara

Thalassaemia International Federation

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From basic research to novel applications on the road of personalized treatment of thalassemia

1st THALAMOSS Scientific Meeting

Ferrara, January 14, 2013
Room E3, Via L.Borsari n.46

Organized by: Department of Life Sciences and Biotechnology, Ferrara University, Italy

Speakers

Sjaak Philipsen (EMC, ERASMUS UNIVERSITAIR MEDISCH CENTRUM ROTTERDAM, Netherlands)
Eitan Fibach (BIOCEP, BIOCEPS, Israel)
Roberto Gambari (UNIFE, UNIVERSITY OF FERRARA, Italy)
Stefano Rivella (CU, CORNELL UNIVERSITY CORPORATION, NY,USA)
Marina Kleanthous, Carsten Lederer (CING, THE CYPRUS FOUNDATION FOR MUSCULAR DYSTROPHY RESEARCH, Cyprus)
Petr Holub (MU, MASARYKOVA UNIVERZITA, Czech Republic)
Matej Lexa (MU, MASARYKOVA UNIVERZITA, Czech Republic)
Frank Grosveld (HA, HARBOUR ANTIBODIES, Netherlands)

THALAMOSS (THALAssaemia MOdular Stratification System for personalized therapy of beta-thalassaemia)

The specific aims of the THALAMOSS Project will be achieved by the development of novel methods for associating variation in genomic data with phenotypic variation. THALAMOSS will combine cutting-edge computing technology with optimized algorithms to mine the unique datasets provided through the proposed project for biologically and medically relevant patterns that can be reliably associated with specific patient groups, treatment response and disease progression. In addition to stratification of patient samples for their molecular properties, the proposed project will also analyse responsiveness to advanced therapeutic approaches to classify patient samples, analysing this responsiveness both as a consequence of molecular properties and as a determinant of the success of novel therapies. At present, the most promising novel approaches to β-thalassaemia treatment are the application of chemical inducers of endogenous foetal haemoglobin (HbF) and transduction of haematopoietic precursor cells with lentiviral vectors expressing exogenous β-globin. In this vein, the proposed project will standardise a high-throughput *in vitro* differentiation protocol of patient-derived erythroid precursor cells and use it to test established inducers of HbF and established β-globin-expressing lentiviral vectors for their therapeutic efficacy and cytotoxicity in a large number of representative cultures from β-thalassaemia patients, in order to identify sample characteristics compatible with palliative chemical or curative gene therapy (GT) intervention. In addition to basic vectors over-expressing β-globin, enhanced vectors (additionally down-regulating disease modifiers and aberrant β-globin mRNA species by an established shRNA co-expression strategy) will be tested in patient samples, in order to assess their potential utility as tools for personalised medicine. Results of these large-scale analyses will have wide-ranging implications for chemical and lentiviral treatments, including the establishment of markers for potentially successful chemical HbF induction and of minimum efficiency requirements for basic and enhanced GT vectors. Taken together, the expected outcome of the THALAMOSS project will be a landmark shift in our approach to the treatment of β-thalassaemia, based on detailed genotype-phenotype correlations, novel markers and a set of standardised analysis procedures for the stratification of patients for optimised disease management and personalised therapy.

Program

8.30-9.00	Registration
9.00-9.10	Welcome
9.10-9.15	**Roberto Gambari** (UNIFE): Introductory remarks
9.15-9.45	**Sjaak Philipsen** (EMC): Molecular control of human fetal globin expression: towards a potential cure for beta-thalassemia and sickle cell disease
9.45-10.10	**Eitan Fibach** (BIOCEP): Purification and culture of thalassemic erythroid precursors
10.10-10.35	**Roberto Gambari** (UNIFE): Inducers of fetal hemoglobin
10.35-11.05	**Stefano Rivella** (CU): Gene transfer and iPS cells for the cure of hemoglobinopathies: expectations, achievements and challenges
11.05-11.30	**Marina Kleanthous, Carsten Lederer** (CING): The ITHANET Community Portal for Thalassaemia and the Haemoglobinopathies
11.30-11.50	**Petr Holub** (MU): Data Management Platform – Challenges ahead of us
11.50-12.15	**Matej Lexa** (MU): Principles and perspectives of genome-wide association studies and related analyses
12.15-12.40	**Frank Grosveld**: Raising normal human antibodies and human heavy chain only antibodies