



# The New Frontier of Precision Medicine - Program

International Summer School University of Pavia - Department of Molecular Medicine

## 3 September - Introduction to the concepts of the Precision Medicine

9:00-9:20 Welcome and presentation of the course (**Luca Arcaini** and **Orsetta Zuffardi** *University of Pavia*)

9:20-10:00 How whole exome and genome sequencing are changing the management of patients (**Raoul Hennekam** *University of Amsterdam*)

10:00-10:40 Some lessons from autoinflammatory diseases (**Alberto Martini** *University of Genoa*)

10:40-11:20 Next generation sequencing: new diagnostic approaches in red blood cell disorders (**Achille Iolascon** *University of Naples Federico II*)

*Coffee break*

11:40-12:20 Current and future gene therapies: from gene silencing to gene editing (**Francesca Forzano** *Guy's and Saint Thomas' NHS Foundation Trust London*)

12:20 13:00 New therapeutic approaches in red blood disorders

*Lunch*

14:00-14:40 Between laboratory and clinic: a continuous conversation (**Orsetta Zuffardi** *University of Pavia*)

14:40-15:20 The Medicine inside the databases (**Enrico Errichiello** *University of Pavia*)

*Coffee break*

15:40-16:00 European Reference Network (ERN): what are and what are the advantages for patients (**Achille Iolascon** *University of Naples Federico II*)

16:00-16:40 From clinical to molecular diagnosis in nephropathies (**Paola Romagnani** *University of Florence*)

16:40-17:20 New therapeutic approaches in nephropathies (**Sabrina Giglio** *University of Florence*)

17:20-18:00 Summary of the day

## 4 September - Precision Medicine and Oncology

9:00-10:20 Hematological malignancies: from genetics to the clinic (**Luca Arcaini** University of Pavia)

*Coffee break*

10:40-12:00 From clinical to molecular diagnosis in myelodysplastic syndromes and their treatment (**Luca Malcovati** University of Pavia)

*Lunch*

13:00-13:40 Monoclonal antibodies and precision medicine (**Ermanno Gherardi** University of Pavia)

13:40-14:20 Benefits and obstacles of immunotherapy in solid tumours (**Vincenzo Russo** San Raffaele University)

14:20-15:00 The role of epigenetics and epigenetic drugs in cancer (**Andrea Mattevi** University of Pavia)

*Coffee break*

15:20-16:00 Liquid biopsy: non invasive monitoring in oncological patients (**Giulia Siravegna** Candiolo Cancer Institute Turin)

16:00-16:40 Personalized approach to advanced NSCLC (**Giulia Stella** University of Pavia)

*Coffee break*

16:50-17:30 Pancreatic cancer: topicality in treatment (**Lorenzo Cobiانchi** University of Pavia)

17:30-18:00 CDx diagnostic companion in personalized therapy of solid tumours (**Matteo Dionisi** Roche Diagnostic)

## 5 September - Precision Medicine, Neurology and Neurodegenerative diseases

9:00-9:40 Parkinson's disease: from genetic diagnosis to genetic treatment (**Enza Maria Valente** University of Pavia)

9:40-10:20 Huntington disease: from genetic diagnosis to genetic treatment (**Mario Pata Roche**)

*Coffee break*

10:40-11:20 Spinal Muscular Atrophy: from hopeless disease to new therapies (**Stefania Corti** *University of Milan*)

11:20-12:00 Duchenne muscular dystrophy and genetic therapy (**Angela Berardinelli** *Mondino Neurological Institute of Pavia*)

Lunch

14:00-14:40 Epilepsies: from molecular diagnosis to precision medicine (**Emilio Perucca** *University of Pavia*)

14:40 15:00 The epileptic encephalopathy from prenatal development to postnatal diagnosis: a paradigmatic case (**A mother accompanied by Dott.ssa Arossa Policlinico San Matteo of Pavia**)

Coffee break

15:20-16:00 New treatments for Alzheimer's disease (**Stefano Govoni** *University of Pavia*)

16:00-18:00 Practises on Databases (**Orsetta Zuffardi** *University of Pavia*) and "Why ethics : a case based training" (**Maria Giovanna Ruberto** *University of Pavia*)

## 6 September - Immunology and Transplantations NIPT lecture

9:00-10:00 New approaches to re-establish tolerance in autoimmune diseases (**Silvia Gregori** *San Raffaele University*)

10:00-11:00 Novel approaches in (xeno)transplantation: CRISPR/Cas9 and genetic engineering for humanized organs, back to the future? (**Roberto Giovannoni** *University of Milan-Bicocca*)

Coffee Break

11:20-12:00 Transplantation ethics (**Maria Giovanna Ruberto** *University of Pavia*)

Lunch

14:00-15:00 Non Invasive Prenatal Testing (NIPT): a revolution in prenatal diagnosis (**Ioris Veermes** *Katholieke Universiteit Leuven*)

16:00-18:00 Practises on Databases (**Orsetta Zuffardi** *University of Pavia*) and "Why ethics : a case based training" (**Maria Giovanna Ruberto** *University of Pavia*)

## 7 September - Hereditary Metabolic Diseases and Precision Medicine Ethics and Precision Medicine

9:00-9:40 The diagnostic approach to inborn errors of metabolism in the era of genomic sequencing (**Nicola Brunetti Pierri** *University of Naples Federico II*)

9:40-10:20 Novel therapies for inborn errors of metabolism (**Nicola Brunetti Pierri** *University of Naples Federico II*)

*Coffee break*

10:40-12:00 Neonatal whole exome sequencing and genetic manipulation : possible related discrimination issues (**Maria Giovanna Ruberto** *University of Pavia*)

*Lunch*

14:00-16:00 Practises on Databases (**Orsetta Zuffardi** *University of Pavia*) and “Why ethics : a case based training” (**Maria Giovanna Ruberto** *University of Pavia*)

## 9-14 September - Workshops

### Methodological approaches to genome for M.D.

Division of participants into small groups according to their main interests in order to prepare a paper the will be presented during the week. Titles and relevant literature will be proposed by the teachers or chosen directly by the students.

Each presentation will be moderated by a professor with the aim of stimulating the discussion among the participants.

The best paper will be rewarded with one of the scholarships made available by the school.

Lectures and exercises on methodological approaches to genome analysis and functional pathogenicity tests, in order to teach how to move into the translational medicine (under the responsibility of **Orsetta Zuffardi** *University of Pavia*).

## 10 September

9:00-13:00 Lecture on methodologic approaches to genome analysis and functional tests: how moving into the translational medicine (**Orsetta Zuffardi** *University of Pavia*)

*Lunch*

14:00-18:00 Workshop activities

## 11 September

9:00-13:00 Lecture on methodologic approaches to genome analysis and functional tests:  
how moving into the translational medicine (**Orsetta Zuffardi** *University of Pavia*)

*Lunch*

14:00-18:00 Workshop activities

## **12 September**

9:00-13:00 Workshop activities

*Lunch*

14:00-18:00 Presentations and discussions

## **13 September**

9:00-13:00 Workshop activities

*Lunch*

14:00-18:00 Presentations and discussions

## **14 September**

9:00-13:00 Presentations and discussions

*Lunch*