



## **COURSE DETAILS**

## **"INTEGRATED COURSE OF**

## **TRANSLATIONAL MEDICINE**"

SSD MEDS-05/A, MEDS-20/A, MEDS-02/B, MEDS-01/A

DEGREE PROGRAMME: MEDICINE AND SURGERY (P11) COORDINATOR: PROF. CARLO GABRIELE TOCCHETTI ACADEMIC YEAR 2024-2025

### **GENERAL INFORMATION – TEACHER REFERENCES**

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# GENERAL INFORMATION ABOUT THE COURSE

TEACHING LANGUAGE: ENGLISH CHANNEL: N/A YEAR OF THE DEGREE PROGRAMME: V SEMESTER: II CFU: 9

# REQUIRED PRELIMINARY COURSES (IF MENTIONED IN THE COURSE STRUCTURE "REGOLAMENTO") NONE

#### **PREREQUISITES (IF APPLICABLE)**

The student must be familiar with the anatomy and physiology of the different organs and systems targeted by therapeutic drugs. Knowledge of the cellular and molecular mechanisms responsible for the main diseases of these organs and systems, and of the homeostatic responses activated by disease states.

#### **LEARNING GOALS**

The aim of the course is to learn advanced knowledge regarding the pathogenesis, the clinical manifestations, the prognostic and therapeutic implications of major chronic diseases.

Students will also be capable to suggest the best therapeutic approach (pharmacologic, or invasive), also considering the benefit-risk ratio and the clinical context.

#### **EXPECTED LEARNING OUTCOMES (DUBLIN DESCRIPTORS)**

Students will acquire the fundamental knowledge that will enable them to understand the underlying mechanisms of major chronic diseases. At the end of the course, students are expected to: 1. know the etiopathogenesis underlying the different systemic diseases; 2. connect morphological aspects to cellular and molecular changes; 3. Recognize direct and indirect symptoms; 4. establish the diagnostic and therapeutic development of the different conditions 5. establish basic indications of the therapeutic strategy (ex: medical or surgical therapy).

#### Knowledge and understanding

The course is mainly focused on the pathophysiology, clinical manifestations and therapeutic management of the main chronic diseases, both malignant and non-malignant.

Discussion of clinical cases will help improving disease knowledge and medical reasoning.

#### Applying knowledge and understanding

Through the theoretical and practical notions provided from the course, students should develop critical thinking skills and should be capable to autonomously deepen their knowledge and be updated on the pathophysiology of major chronic diseases.

At the end of the course, students should fluently discuss all the learned notions, using the most appropriate terminology and should also be capable to communicate, with a simple but comprehensive and accurate language, the main information about major chronic diseases to non-experts (i.e. patients)

#### **COURSE CONTENT/SYLLABUS**

- 1. CARDIAC FUNCTION AND DYSFUNCTION
- 1.1 Cellular Biology of the heart
- 1.2 The Heart and other systems:
- 1.3 Principles of Cardioncology
- 1.4 Heart and Immunity
- 1.5 Heart and Kidney
- 2. CARDIOPULMONARY INTERACTIONS
- 2.1 Pathology and Pathobiology of pulmonary vascular diseases
- 2.2 Pulmonary Hypertension
- 2.3 Acute and chronic Right Heart Failure in Pulmonary diseases
- 3. VULNERABILITY AND FRAILTY
- 3.1 The multidimensional phenotype
- 3.2 Chronic conditions

- 4. PRINCIPLES OF HAEMOSTASIS
- 4.1 Bleeding disorders
- 4.2 Thrombotic disorders
- 4.3 Antithrombotic and antihaemorragic approaches

#### 5. CARDIOVASCULAR PREVENTION

- 5.1 Hypertension
- 5.2 Dyslipidemia
- 5.3 Ischemic heart disease
- 5.4 Cerebrovascular diseases

#### 6. CARDIOVASCULAR AGEING AND THE ADRENERGIC SYSTEM

- 6.1 Regenerative Therapy in Heart Failure: stem cell-based
- 6.2 Regenerative Therapy in Heart Failure: gene therapy
- 6.3 Adrenergic nervous system in heart failure: from bench to bedside
- 6.4 Adrenergic nervous system in physiological ageing

#### 7. THERAPEUTIC APPROACHES TO GENETIC DISEASES

- 7.1 The revolution of next generation sequencing in medical genetics
- 7.2 Precision medicine (the example of cystic fibrosis)
- 7.3 Genetics goes translational PIK3CA-Related Overgrowth Spectrum (PROS) and SMA
- 7.4 Prenatal diagnosis and genetic counseling

#### 8. THERAPEUTIC APPROACHES TO GENETIC DISEASES

- 8.1 Principles and applications of gene therapy I (viral vectors and various gene therapy approaches including genome editing)
- 8.2 Principles and applications of gene therapy II (successful applications of ex vivo and in vivo gene therapy)
- 8.3 Translating gene therapy from bed to bedside: the example of Luxturna (from pre-clinical to Phase III and market approval)
- 8.4 Pharmacological, protein and cellular therapies for genetic diseases
- 9. DIABETES
- 9.1 Principles of glucose homeostasis and hormonal regulation
- 9.2 Perspectives into the Molecular Pathogenesis of Type 2 Diabetes
- 9.3 Type 2 Diabetes management and therapeutic advances
- 9.4 Update for real-world translation of interventions for Type 2 Diabetes prevention
- 10. ONCO-IMMUNOLOGY
- 10.1 Precision Cancer Medicine
- 10.2 Models in translational oncology
- 10.3 Rational drug development
- 10.4 Focus on Immuno-Oncology

#### 11. PEDIATRIC TRANSLATIONAL MEDICINE

- 11.1 ImmunoNutrition
- 11.2 GI disorders
- 11.3 Obesity and Metabolic Syndrome
- 11.4 Allergy and Immunology

- 12. GENETICS IN PEDIATRIC DISEASES
- 12.1 The Genetic approach in pediatric medicine
- 12.2 Patterns of genetic transmission
- 12.3 Genetic approaches to rare and undiagnosed diseases
- 12.4 Inborn errors of metabolism: diagnosis and treatment
- 13 STATISTICS IN TRANSLATIONAL MEDICINE
- 13.1 Statistics in the Critical Evaluation of Biomedical Scientific Literature
- 13.2 Critical Evaluation of Study Designs in Biomedical Research
- 13.3 Assessing Methodological Quality and Statistical Rigor
- 13.4 Interpreting Statistical Results in Biomedical Research

TEACHING ACTIVITIES				
Week	Day; Time	Lesson	Professor	
1° 3-7 March	Monday March 3; 13.00-14.00	Principles and applications of gene therapy I (viral vectors and various gene therapy approaches including genome editing)	Trapani	
2025	Monday March 3; 14.00-15.00	Principles and applications of gene therapy II (successful applications of ex vivo and in vivo gene therapy)	Trapani	
	Monday March 3; 15.00-16.00	The Role of Statistics in the Critical Evaluation of Biomedical Scientific Literature	Dolce	
	Monday March 3; 16.00-17.00	Critical Evaluation of Study Designs in Biomedical Research	Dolce	
2° 10-14	Monday March 10; 13.00-14.00	Systemic and Pulmonary Vascular Diseases: Pulmonary Hypertension	Mercurio	
March 2025	Monday March 10; 14.00-15.00	Cardio-pulmonary Diseases: Acute right Heart Failure	Mercurio	
	Monday March 10; 15.00-16.00	Principles of the haemostatic balance	Di Minno	
	Monday March 10; 16.00-17.00	Bleeding disorders	Di Minno	
3°	Monday March 17; 13.00-14.00	Cardio-pulmonary Diseases: Chronic right Heart Failure	Mercurio	
17-21 March 2025	Monday March 17; 14.00-15.00	Cardio-pulmonary Diseases' therapy: Two Sides of the Same Coin.	Mercurio	
	Monday March 17; 15.00-16.00	The revolution of next generation sequencing in medical genetics	Trapani	
	Monday March 17; 16.00-17.00	Precision medicine (the example of cystic fibrosis)	Trapani	
4° 24-28	Monday March 24; 13.00-14.00	Adrenergic nervous system in heart failure: from bench to bedside	Rengo	
March 2025	Monday March 24; 14.00-15.00	Guidelines to manage chronic diseases: the example of acute and chronic heart failure	Rengo	
	Monday March 24; 15.00-16.00	Diseases' interaction: the example of ischemic preconditioning	Tocchetti	
	Monday March 24; 16.00-17.00	Diseases' interaction: the example of cardio-immunology	Tocchetti	
5° 31 March –	Monday March 31; 13.00-14.00	From bench to bedside: examples of pharmacological clinical studies	De Rosa	
4 April	Monday March 31; 14.00-15.00	Pediatric ImmunoNutrition	Berni Canani	
2025	Monday March 31; 15.00-16.00	Diseases interaction: the example of cardio-oncology	Tocchetti	
	Monday March 31; 16.00-17.00	Diseases interaction: the example of cardio-renal syndrome	Tocchetti	
6° 7–11 April	Monday April 7; 13.00-14.00	Translating gene therapy from bench to bedside: the example of Luxturna (from pre-clinical to Phase III and market approval)	Trapani	
2025	Monday April 7; 14.00-15.00	Pharmacological, protein and cellular therapies for genetic diseases	Trapani	
	Monday April 7; 15.00-16.00	Thrombotic diseases	Di Minno	
	Monday April 7; 16.00-17.00	Antithrombotic and antihemorrhagic approaches	Di Minno	
7	Monday April 14; 13.00-14.00	From Goldblatt's model to hypertensive patient	De Rosa	
14 - 18	Monday April 14; 14.00-15.00	Pediatric GI Diseases	Berni Canani	
April 2025	Monday April 14; 15.00-16.00	Genetics goes translational PIK3CA-	Trapani	

		Related Overgrowth Spectrum (PROS) and SMA		
	Monday April 14; 16.00-17.00	Prenatal diagnosis and genetic counseling	Trapani	
9°	Monday April 28; 13.00-14.00	Atherosclerotic Vulnerable Plaque: the "Sacred Graal"	De Rosa	
28 April – 2	Monday April 28; 14.00-15.00	Dyslipidemia: to treat or not to treatthat is the question	De Rosa	
May 2025	Monday April 28; 15.00-16.00	General approach for the diagnosis of genetic disorders	Parenti	
	Monday April 28; 16.00-17.00	Methods for genomic analysis	Parenti	
10° 5-9 May	Monday May 5; 13.00-14.00	Alternative therapy approach in chronic diseases: the example of stem cells and gene therapy in Heart Failure	Rengo	
2025	Monday May 5; 14.00-15.00	Physical activity as a drug in chronic disease: the example of Heart Failure	Rengo	
	Monday May 5; 15.00-16.00	Assessing Methodological Quality and Statistical Rigor	Dolce	
	Monday May 5; 16.00-17.00	Interpreting Statistical Results in Biomedical Research	Dolce	
11	Monday May 12; 13.00-14.00	Pediatric Obesity and Metabolic Syndrome	Berni Canani	
12 – 16	Monday May 12; 14.00-15.00	Pediatric Allergy and Immunology	Berni Canani	
May 2025	Monday May 12; 15.00-16.00	New therapies for Inborn errors of metabolism	Parenti	
	Monday May 12; 16.00-17.00	Clinical trials	Parenti	
12	Tuesday May 19; 13.00-14.00	Precision Cancer Medicine	Malfitano	
19 – 23	Tuesday May 19; 14.00-15.00	Models in translational oncology	Malfitano	
May 2025	Tuesday May 19; 15.00-16.00	Principles of glucose homeostasis and hormonal regulation	Raciti	
	Tuesday May 19; 16.00-17.00	Molecular Pathogenesis of Type 2 Diabetes	Raciti	
13	Monday May 26; 13.00-14.00	Rational drug development	Malfitano	
26-30 May	Monday May 26; 14.00-15.00	Focus on Immuno-Oncology	Malfitano	
2025	Monday May 26; 15.00-16.00	Type 2 Diabetes management and therapeutic advances	Raciti	
	Monday May 26; 16.00-17.00	Interventions for Type 2 Diabetes prevention	Raciti	

#### **READINGS/BIBLIOGRAPHY**

#### RECOMMENDED TEXTBOOKS AND DIDACTIC MATERIAL

- HARRISON'S PRINCIPLES OF INTERNAL MEDICINE
- Nelson textbook of pediatrics 21st edition
- Thompson and Thompson Genetics in Medicine
- Martin Bland, An Introduction to Medical Statistics (4th Edition)

Any other didactic material recommended by the Professors

#### **TEACHING METHODS**

Exam type:

The course will be based on frontal teaching (lectures with the support of power point presentations) providing 6 CFU and clinical clerkships providing another 3 CFU.

#### **EXAMINATION/EVALUATION CRITERIA**

#### a)

For *integrated courses*, there should be one exam.

Exam type	
written and oral	

only written	
only oral	Х
project discussion	
other	

In case of a written exam, questions refer	Multiple choice answers	
to: ( <sup>*</sup> )	Open answers	
	Numerical exercises	

(\*) multiple options are possible

# b) Evaluation pattern:

the oral exam consists of at least 2-3 questions spanning among the 4 SSDs