

Course unit English denomination	The world of RNA: from cell biology to personalized medicine
SS	06/MEDS-02
Teacher in charge (if defined)	Prof. Rosario Rizzuto, Dott.ssa Annamaria Lia
Teaching Hours	10
Number of ECTS credits allocated	2
Course period	To be defined
Course delivery method	☑ In presence☐ Remotely☐ Blended
Language of instruction	English
Mandatory attendance	e ⊠ Yes (80% minimum of presence) □ No
Course unit contents	1. Introduction to the world of RNA 2. RNA Delivery: strategies and limitations 3. mRNA: applications 4. RNA interference and ASOs 5. siRNA: advantages and examples
Learning goal	This course aims to provide students with basic knowledge of the world of RNA and the concept of personalized medicine. The course will cover RNA drug delivery challenges (stability, degradation and immune response) and strategies (lipid nanoparticles, viral vectors, and polymer-based systems). Covid-19 mRNA vaccine delivery will be presented as an example. In addition, basic knowledge of the various types of RNA will be provided. As for mRNA both vaccines and proteins replacement therapy will be discussed. Gene silencing approaches mediated by RNA interference and ASOs will be

At the end of the course, students will be able to:

- Understand the structural and functional properties of RNA.
- Analyze various RNA delivery strategies and their limitations.

presented. Some examples of FDA-approved RNAi or ASO-based therapies will be discussed, such as the case of Inclisiran for hypercholesterolemia and Eteplirsen for Duchenne muscular dystrophy. Finally, also the advantages of siRNA with respect to other strategies will be examined. As a case-study will be presented the case of Patisiran for hereditary transthyretin amyloidosis.

• Evaluate the therapeutic applications of mRNA and its role in modern medicine.





- Examine RNA interference (RNAi) and antisense oligonucleotides (ASOs) as gene-silencing tools.
 Understand the advantages and applications of siRNA therapeutics.

Teaching methods	The course is divided into 5 frontal lessons.
Course on transversal interdisciplinary, transdisciplinary skills	' □ Yes ☑ No
Available for PhD students from other courses	\boxtimes Yes Classes are open to doctoral students only in the following courses: Biosciences \square No
Prerequisites (not mandatory)	
Examination methods (if applicable)	Test multiple choice.
Suggested readings	Bibliographical references for further study will be provided during the course.
Additional information	