Finanziato dall'Unione europea NextGenerationEU
Ministero dell'Università e della Ricerca
Italiadomani PIANO NAZIONALE DI RIPRESA E RESILIENZA
UNIVERSITÀ DEGLI STUDI DI NAPOLI FEDERICO II

PNRR MUR

Missione 4, Componente 2, Investimento 1.4 "Potenziamento strutture di ricerca e creazione di "campioni nazionali di R&S" su alcune Key Enabling Technologies"

Iniziativa finanziata dall'Unione europea –- NextGenerationEU.

National Center for Gene Therapy and Drugs based on RNA Technology
Sviluppo di terapia genica e farmaci con tecnologia a RNA

Codice progetto MUR: **CN00000041** – CUP UNINA: **E63C22000940007**

National PhD Program
RNA THERAPEUTICS AND GENE THERAPY

PLANNED TEACHING ACTIVITY

Scheduled Courses – I year

n.	Course name	Total amount of hours	Course Description	Study programme
1.	Digital skills	6	The course provides an introduction to Big Data Science with guided hands-on. In particular, there will be an overview of statistical methods used in biological and medical research. Emphasis will be put on applications to real problems.	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
2.	Design and validation of reporter systems for preclinical drug delivery and RNAi efficiency	6	The course describes reporter systems for monitoring drug delivery and RNAi efficiency in preclinical studies and how they allow for the visualization and quantification of gene expression, protein production, and other cellular processes in living cells and animal models. The course teaches how to select appropriate reporter genes, design constructs for efficient delivery, and test their responsiveness to the targeted drug or RNAi. Practical application of validated reporter systems to get insights into drug delivery mechanisms and RNAi efficiency, optimize drug development and screening are provided.	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
3.	Gene Therapy with DNA and RNA	6	The objective of this course is to provide an in-depth overview of the challenges and opportunities of gene therapy using DNA and RNA. Therapy design, choice of vectors. Development of therapies and their testing in vitro and in vivo in different animal models of cardiac diseases. How to move a therapy from bench to bedside is discussed with the participation of scientists who "have done it".	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
4.	Delivery of nucleic acid therapeutics with nanotechnologies	6	The course provides an overview of drug delivery systems for local and systemic administration of RNA and DNA therapeutics referring to biological rules for rational design, formulation, production, and quality requirements. Issues such as biocompatibility and nanotoxicology are considered too. The course also deals with the regulatory framework to develop nanomedicines. Specific examples of how to develop nanoplatforms for precision delivery to solid tumors are provided.	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES

Scheduled Courses – II year

n.	Course name	Total amount of hours	Course Description	Study programme
1.	Toxicology and Immunogenicity of RNA drugs	6	The course provides a comprehensive overview on the preclinical tools to study the potential side effects associated with RNA drugs (and their vehicle) with a special emphasis on unwanted immune responses effects	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
2.	Regulatory aspects of RNA and gene therapies	6	This course provides an overview on drug regulation as a key issue for the clinical translation of RNA drugs and gene therapies. The different topics that are characterizing the ongoing discussion on the regulation of these new drug classes are faced.	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
3.	Smart drug delivery systems	6	The course aims at introducing the features expected from advanced nanocarriers for drug delivery, the properties affecting their biological behaviour, the rational for design of "smart" nanocarriers to exploit the phenotypic and microenvironmental features of the tumor to increase site-selectivity	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
4.	Human relevant preclinical models generation and characterization	6	The course provides knowledge useful for the identification and development of human-relevant models for the study of RNA drugs therapeutic activity in preclinical studies (cell cultures, organoids, in embryo). Innovative methodologies such as reporter cells and animals engineered to express easily measurable readout in vivo (e.g. by bioluminescence, PETR, NMR imaging) will be applied to create rapid, reproducible and high throughput screening assays.	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES

Scheduled Courses – III year

n.	Course name	Total amount of hours	Course Description	Study programme
1.	Multiomics approaches for drug discovery	6	The course provides a comprehensive overview on Genomics, Transcriptomics, Proteomics, Metabolomics and Lipidomics. Both the theoretical and technological aspects of different omic-sciences will be overviewed. The course will be focused on NGS technology for genome and transcriptome analysis for identifying therapeutic targets and on the application of advanced MS-based approaches for proteomics, metabolomics and lipidomics.	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES
2.	Bioinformatics	6	The course provides a comprehensive overview on existing methods and software tools for understanding biological data, when the data sets are large and complex. The course introduces the basic concepts of Data Mining (classification, clustering, associative rules) and the main data preprocessing techniques and describes how to set up an "in silico" data analysis experiment using different platforms or	**1.** DESIGN AND DELIVERY OF NEW GENE THERAPY AND RNA-BASED MEDICINES; **2.** MECHANISMS OF DISEASES AND DRUG TARGET IDENTIFICATION; **3.** VALIDATION AND SAFETY IN PRECLINICAL AND CLINICAL STUDIES

			languages, and some public access omics and clinical datasets.	