

PNRR 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**

Doctorate of National Interest
RNA THERAPEUTICS AND GENE THERAPY

TITLE OF THE RESEARCH PROJECT

Integrated MS-omics approaches for drug target identification and mechanism elucidation.

SELECT ONE OF THE FOLLOWING RESEARCH AREA:

- ☒ **Mechanisms of Diseases and Drug Target Identification**
- ☐ **Design and Delivery of New Gene Therapy and RNA-Based Medicines**
- ☐ **Validation and Safety In Preclinical and Clinical Studies**

LOCATION OF THE RESEARCH ACTIVITY (INSTITUTION/DEPARTMENT):

University of Salerno, Department of Pharmacy

TUTOR: Prof. Pietro Campiglia, Co-TUTOR Prof Eduardo Maria Sommella

PROPOSED RESEARCH ACTIVITIES (max 300 words):

To minimize issues during preclinical or clinical trials, it is crucial to establish the in vivo efficacy and toxicity of any drug candidate early in the drug discovery process. Typically, lack of translational efficacy stems from inadequate knowledge of the drug's mechanism of action, while adverse effects usually result from abnormal pharmacokinetics and synergistic or off-target effects. The employment of multi-omics approaches, eg the combination of metabolomics, lipidomics and proteomics, early in drug development is particularly valuable, as they can generate early, specific, and translational metabolite, lipid or protein biomarkers or signatures for both efficacy and side effects. Additionally, these studies provide a comprehensive understanding of drug action and potential toxicity mechanisms. The objective



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FEDERICO II

of this project is to integrate multiple omics approaches using state-of-the-art hyphenated mass spectrometry techniques. The project will apply these integrated omics methods to cellular models, organoids, and biofluids and tissues from in vivo preclinical studies. This approach aims to elucidate the molecular effects of novel drugs and their formulations, uncover new molecular insights, facilitate target deconvolution, and highlight key pathways to better understand pathophysiology and drug mechanisms.