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**

SPOKE 9: From target to thrapy and competence center

Validating acid nucleic-based drugs using in vitro and in vivo models of cancer.

Molecular therapies and precision medicine represent a new era of modern pharmacology *(Sadee at al., Pharmacological Reviews July 2023, 75 (4) 789-814)*. The need for highly specific new drugs stems from the astounding advancements in the understanding of molecular and cellular events underlying the pathogenesis of human diseases including cancer *(Hoeben et a., Cancers Basel 2021 Jan; 13(2): 242)*. It has become clear that neoplastic diseases with similar clinical and phenotypical progression, differ from each other for genomic and/or genetic causes including the alteration of oncogenes and tumor suppressor genes. For this reason, currently, innovative approaches able to specifically target altered genes or proteins show high efficacy *(Ho Shin et al., npj Precision Oncology (2017) 1:12)*. In this regard, the remarkable efficacy, safety and flexibility of nucleic acid-based drugs promise to forever change the landscape of cancer therapy. Despite this, there is very limited information and methodologies available about the pharmacokinetics, pharmacodynamics and toxicology of these innovative drugs. For this reason, the project aims to develop science-based methodologies to shed light on the distribution, metabolism, pharmacological activity and potential side effects of nucleic acid-based drugs. To achieve this goal nucleic acid-based drug therapies will be evaluated and validated in *in vitro* and *in vivo* cancer experimental models.