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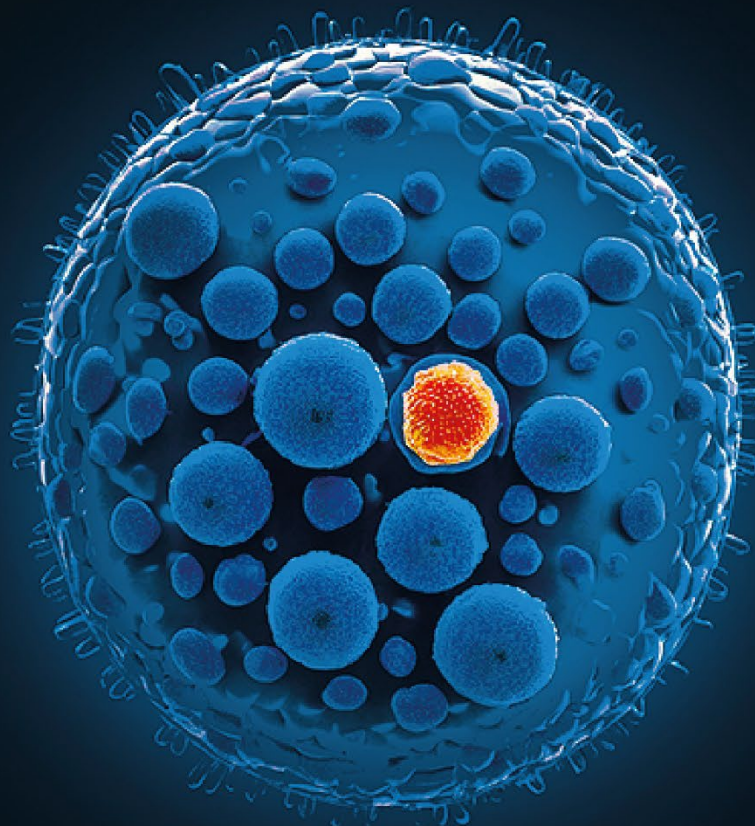


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e della Ricerca



Italiadomani
PIANO NAZIONALE
DI RIPRESA E RESILIENZA

HEAL ITALIA



FORUM NAZIONALE SULLA MEDICINA DI PRECISIONE

Il Modello HEAL ITALIA e il contributo della Ricerca al Sistema Sanitario del Futuro

PALERMO

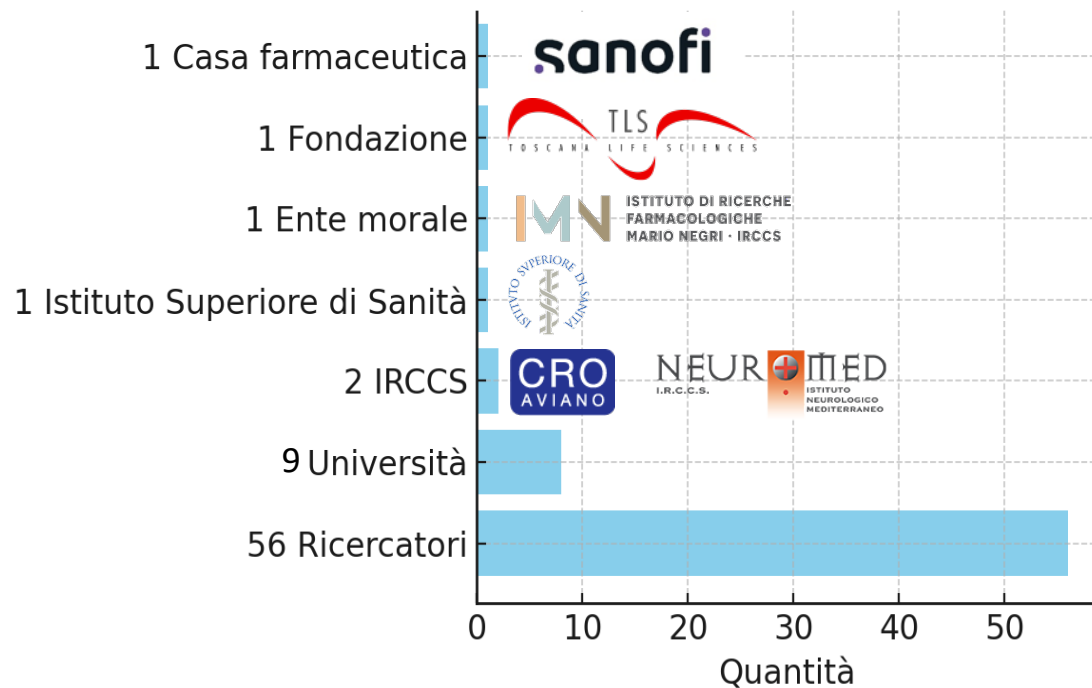
13 · 14 · 15

GIUGNO 2024

SPOKE 5: Next-gen Therapeutics

○ SPOKE PRESENTATION

- **56 researchers involved: 40 PIs, 8 newly recruited RTD-A, 2 newly recruited ARs, 3 newly recruited PhD students (+ 3 Fellows and 1 PhD)**



sanofi

TLS
TOSCANA LIFE SCIENCES

IMN
ISTITUTO DI RICERCHE FARMACOLOGICHE MARIO NEGRI - IRCCS

ISTITUTO SUPERIORE DI SANITÀ

CRO AVIANO

NEUR
I.R.C.C.S.

IMED
ISTITUTO NEUROLOGICO MEDITERRANEO

UNIVERSITÀ DEGLI STUDI DI MILANO
BICOCCA

UNIMORE
UNIVERSITÀ DEGLI STUDI DI MODENA E REGGIO EMILIA



Università degli Studi di Palermo



UNIVERSITÀ di VERONA



SAPIENZA
UNIVERSITÀ DI ROMA

UNIVERSITÀ di CATANIA



UNIVERSITÀ DI PISA



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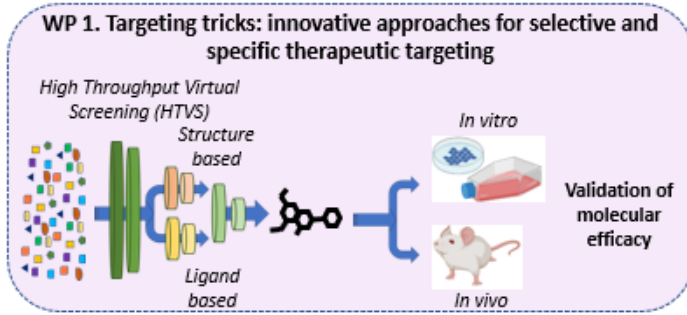
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SPOKE 5: Next-gen Therapeutics

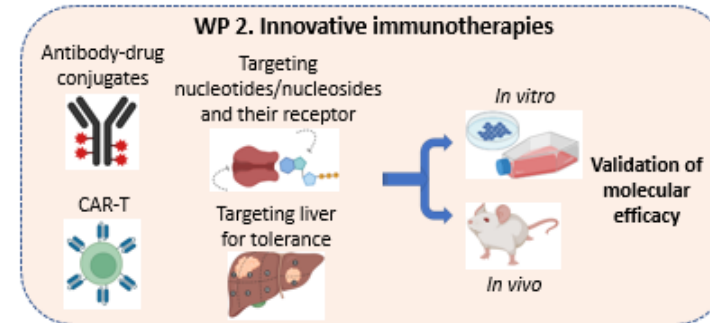
From silico to bedside” design and validation of innovative tailored and personalized therapeutic strategies

UNIPA, UNIMIB



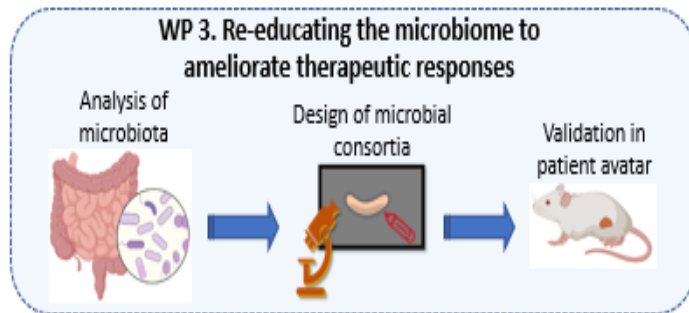
AIM: To develop successful therapeutic strategies for genetic diseases due to non-sense mutations or mutations causing functional defects

UNIMIB, CRO, TLS, UniRoma1, UNIMORE, UNIPA, UNICA, UNIVR



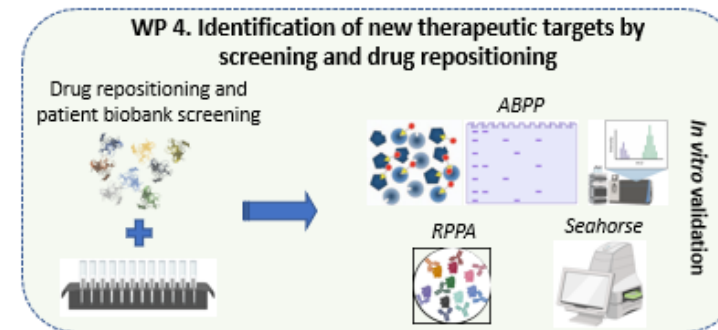
AIM: To generate tools for the selective killing or control of pathogenic cells with high treatment efficacy, containing side effects and reduce the time of therapy administration

UNIMIB, UNIBO, UNICT, UNIMORE, UNIFIG, SANOFI, Neuromed



AIM: To identify innovative microbiota modulating therapies tailored on the individual patient’s microbial dysbiosis.

SAPIENZA, UNIMORE, IFO, ISS, UNIBO, UNIMIB, UNIROMA1, M. NEGRI

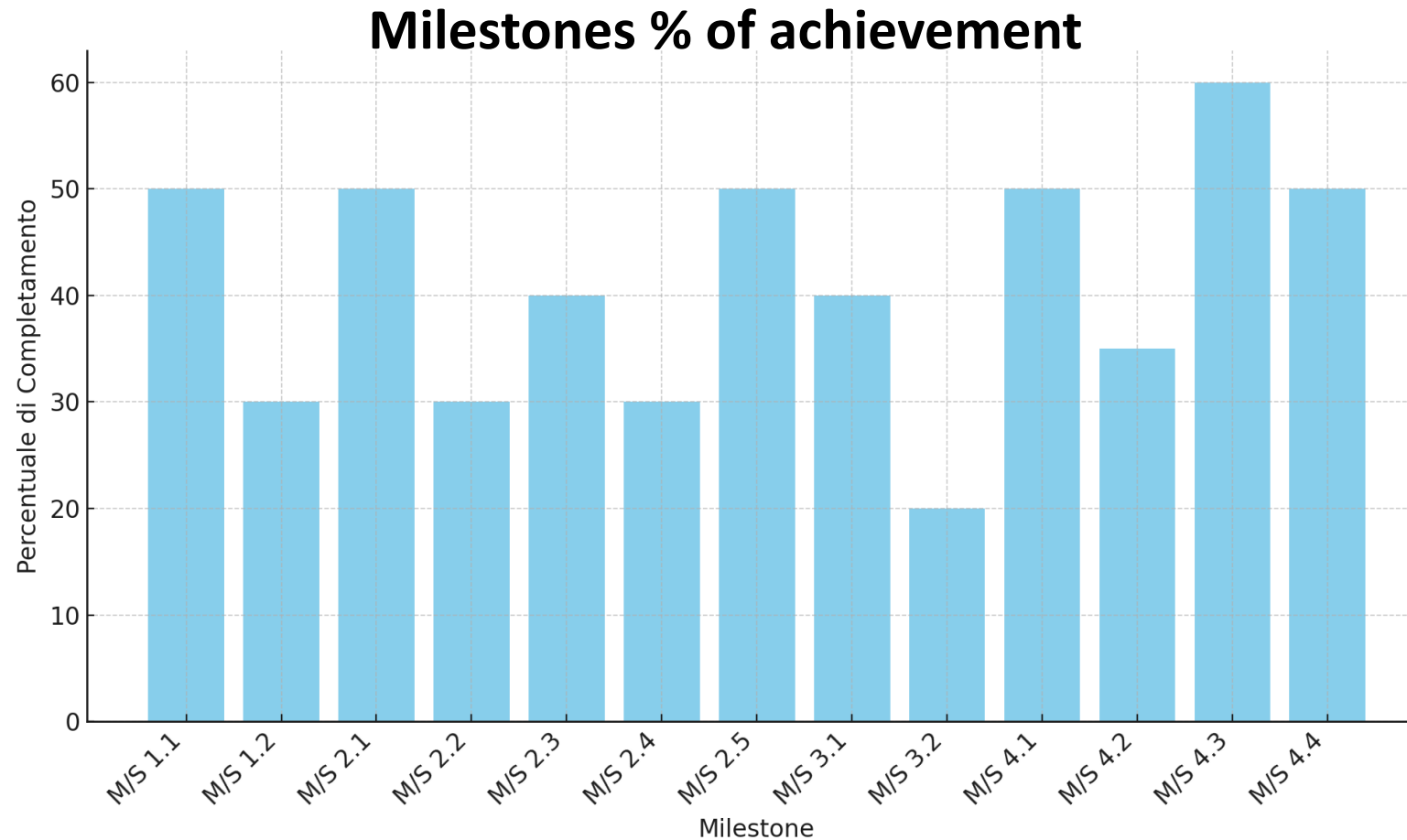


AIM: To optimize a pipeline for the identification of novel candidate drugs and testing of available drugs for human diseases, using modeling tools and AI.

SPOKE 5

Next-gen Therapeutics

○ WHERE WE ARE



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HEAL **ITALIA**

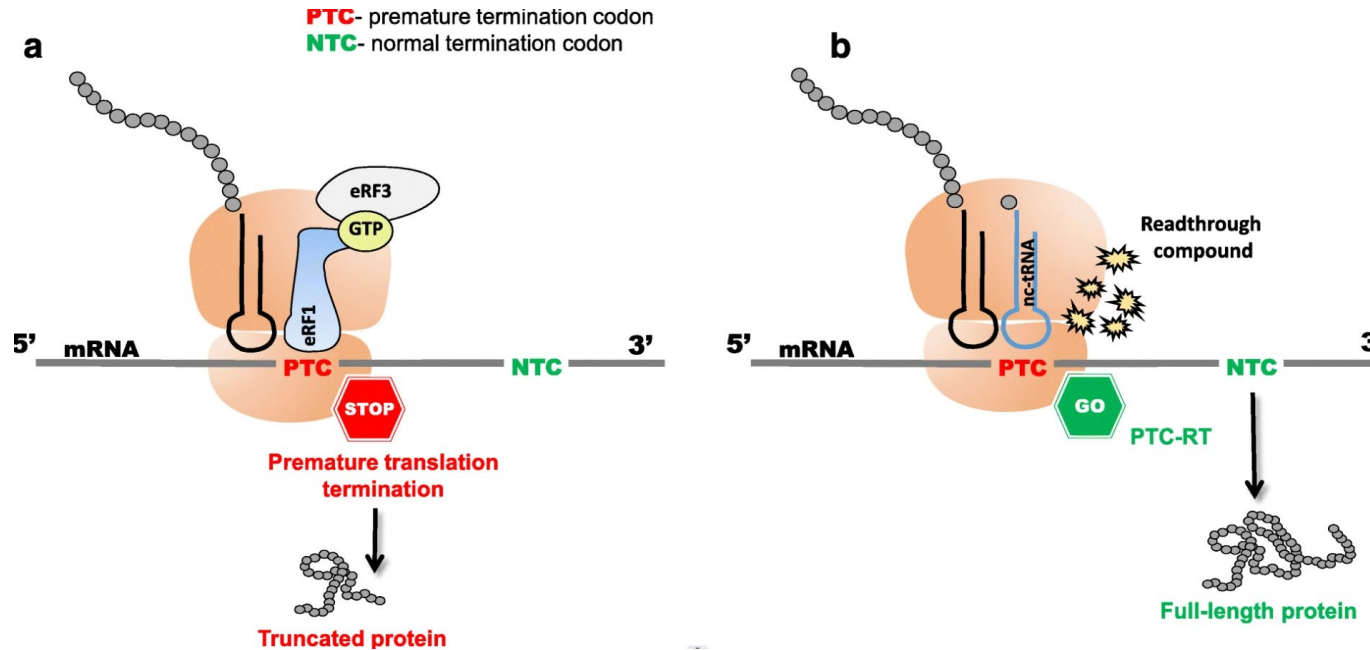
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○ WHERE WE ARE GOING

WP1: Genetic Diseases

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New TRIDs identified for the restoration of CFTR expression in a new mouse model of Cystic Fibrosis



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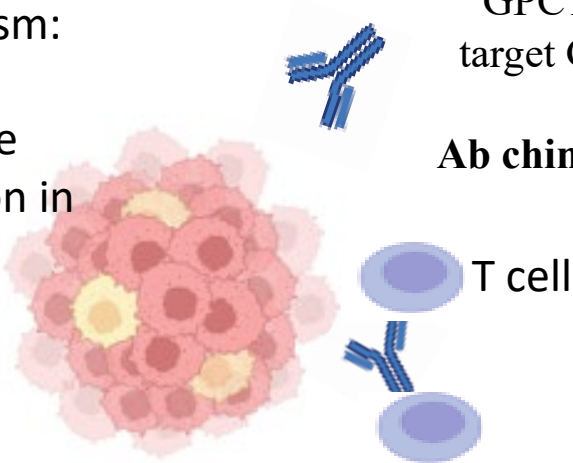
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WHERE WE ARE GOING

WP2: Tumors, autoimmune diseases

Metabolism:
reduce
adenosine
generation in
the TME



GPC1, GPC3, GD2 and CD138 to
target GBM, PDAC; HCC and MM

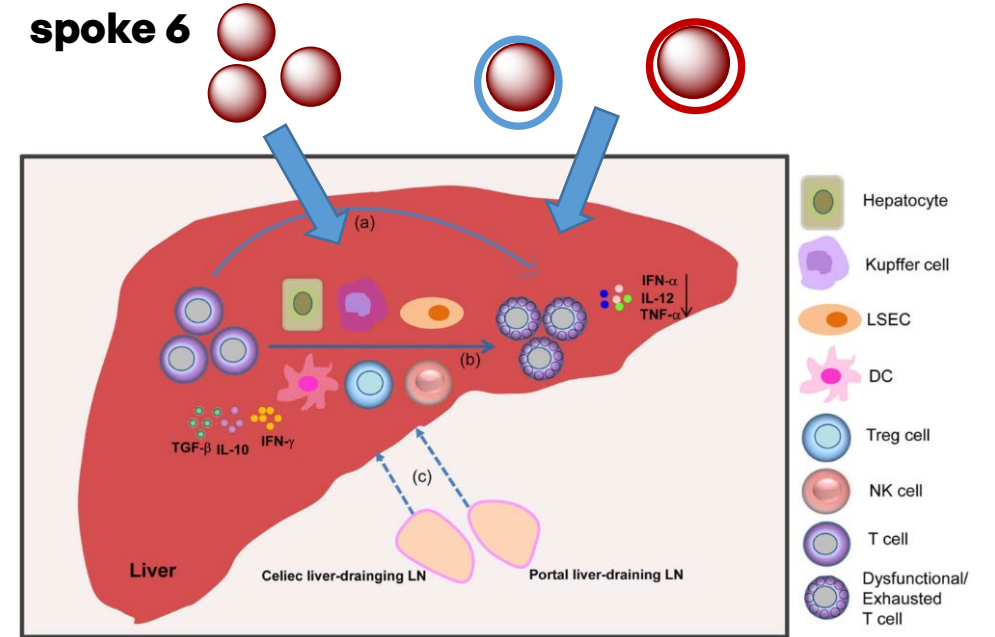
Ab chimeric collaboration spoke 8

CAR-T or Bi-specifics CAR-T

Tumors

Nanoparticles collaboration spoke 6

Antigen transfer to the liver to induce tolerance



Autoimmune diseases



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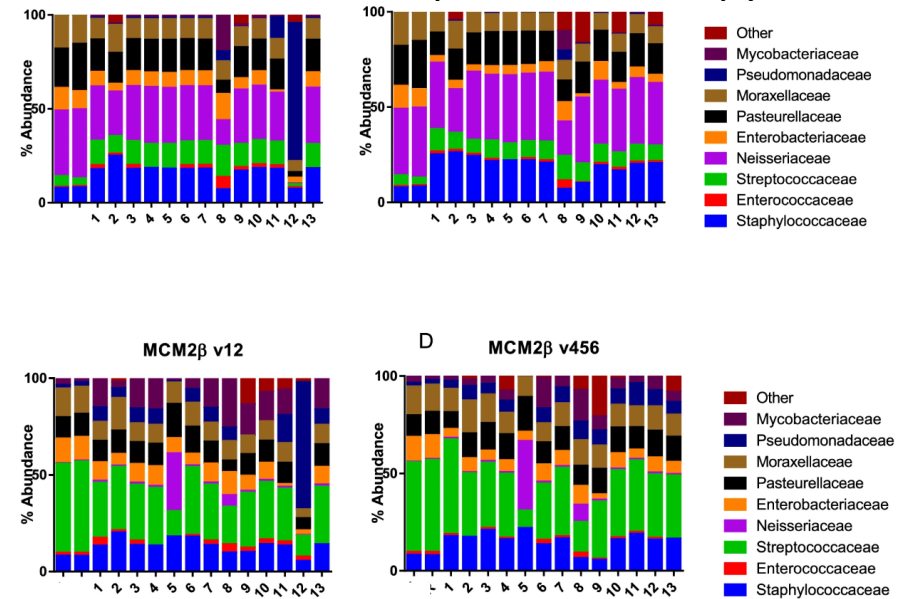
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○ WHERE WE ARE GOING

WP3 : Microbiota, dysbiosis



Characterization of the microbiota in different dysbiosis conditions, correlation with disease and response to therapy

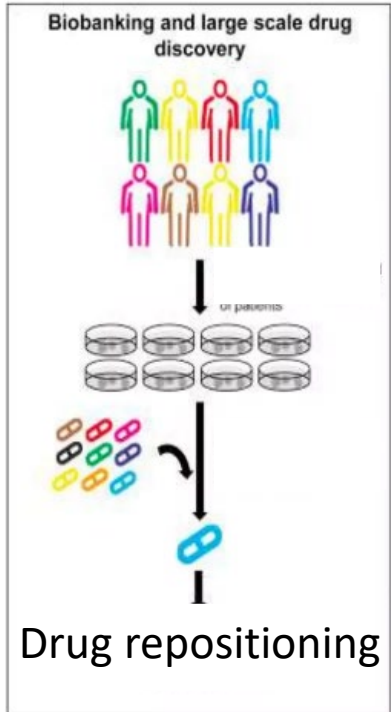


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○ WHERE WE ARE GOING

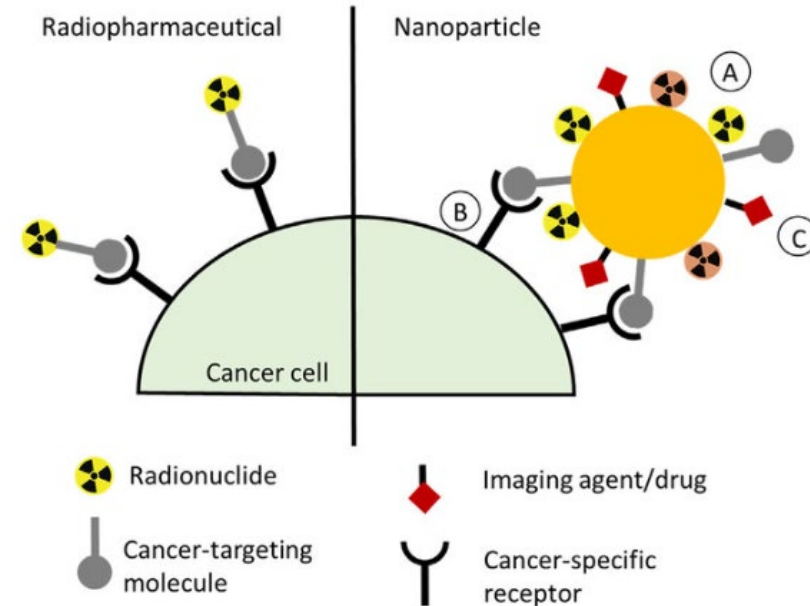
- **WP4:** Drug repurposing per PID, NAFLD/NASH; new radiopharmaceuticals



Generation and Screening of Biobanks (PID and NAFLD/NASH)

Screening of existing biobanks
Collaboration with spoke 1, 2,3

Drug repurposing



Radiopharmaceuticals against tumors

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○ THE CHALLENGE

- **Where we will be in month 36**
- **WP1:** new molecules correcting premature translation termination and protein misfolding
- **WP2:** new biological drugs and nanodrugs for the treatment of tumors and autoimmune diseases; definition of metabolic pathways on which to intervene pharmacologically to obtain immunomodulatory activity
- **WP3:** Identification of dysbiosis conditions that influence anti-tumor therapies; of microbiota components with immunomodulatory effect; of microbiota components negatively modulated by chronic drug use
- **WP4:** new molecules identified in silico for the regeneration of cardiomyocytes and for use as anticancer agents; of new radiolabeled drugs for anti-tumor therapy; «repurposed drugs» for the therapy of NASH/NAFLD or rare PID diseases with gastrointestinal involvement



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Next-gen Therapeutics

○ FORECASTS AFTER THE CLOSURE OF THE PROJECT

Search for Financing : Garantire la continuità delle attività di ricerca e sviluppo.

Collaborations and Partnerships : New collaborations with research bodies, universities and pharmaceutical companies to exploit synergies and accelerate the implementation of therapies.

Commercialization and Technology Transfer : Transfer the technologies developed from the laboratory to the market.

Development of Future Research Lines : Identify and develop new lines of research based on discoveries made during the project.

Formazione : Train the next generation of researchers and specialists in the field of innovative therapies.



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