The National Center on Gene Therapy and Drugs based on RNA Technology

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Finanziato dall'Unione europea
NextGenerationEU

MUR
Ministero dell'Università e della Ricerca

Rappresentanza Permanente d'Italia
Organizzazioni internazionali - Parigi

Italian Research Day in the World
(14 April 2023, OECD, 2 rue André Pascal, 75116 Paris
Conference Centre Room CC13)

giornata della RICERCA ITALIANA

Rappresentanza Permanente d'Italia
UNESCO

THE CONTEXT

Diseases of low prevalence are often neglected by profit-based drug development
Molecular understanding of diseases divides patients in subgroups that benefit from targeted drugs (personalized medicine)

Progress in clarification of diseases pathogenesis and technological advancement open new therapeutic perspectives
- Gene therapy (gene correction by CRISPR-CAS9, CAR-T)
- Depevelopment of RNA as «universal» therapeutic molecule (mRNA, siRNA, microRNA)

Scientific and technological knowledge allows not only economic exploitation of scientific progress, but also equality in cure and sustainability of universal health systems

THE REQUIREMENT OF THE CALL

The Center initiates the process of transforming scientific knowledge in the development of therapies or procedures of pharmacological interest, testing them in clinical trials aimed at demonstrating their safety and potential efficacy (proof of principle). In this context, the Center facilitates investment in sectors with high economic risk for the country by start-ups, biotech and large industry. The Center focuses its activities on areas of high innovative value, or not necessarily priority for Big Pharma, such as gene therapy applied to the treatment of cancer or hereditary diseases and RNA-based technologies, integrating advanced biocomputing skills and smart nanomaterials. In the strategic areas selected, the Centre has the ambition and the ability to become an excellence and a point of reference for Europe in order to make our country competitive in the development of cutting-edge medicines.

1. Transfer of fundamental research into drug development
2. Tech transfer in highly innovative sectors
3. Social impact (fairness of health system)

THE EXPECTED OUTCOME

Business

Building the pipeline from research on disease pathogenesis to production of innovative drugs;

Making the Country competitive in “pathogenesis-driven” drug design;

Connect the widespread knowhow of the Country, with a global national effort and special attention to the Southern regions

Society

Developing the drugs necessary for precision medicine, guaranteeing the sustainability of the health system and the treatment of rare diseases, neglected by profit-based companies;

Reducing the gaps, not only geographic but also of gender and age, thus expanding the human capital (training and access to labour market of young and women, brain gain from abroad)

3 PILLARS

1. Improving and rooting in the nation the KET
    - NA chemistry (including molecular modelling)
    - precision delivery (nanoparticles, viral vectors)
    - efficacy, toxicity and pharmokinetics studies
2. Developing new drug design projects: from research to drug
    - genetic diseases
    - cancer
    - neurodegenerative diseases
    - metabolic and cardiovascular diseases
    - inflammatory and infectious diseases
3. Bringing to clinics an increasing number of AGTMP
    - Gene Therapy Consortium (OPBG, TIGET, Tettamanti Foundation)

HUB AND SPOKE ORGANIZATION: 10 THEMATIC SPOKES

▶ VERTICAL SPOKES

Genetic diseases
Cancer
Neurodegenerative diseases
Metabolic and cardiovascular diseases
Inflammatory and infectious diseases

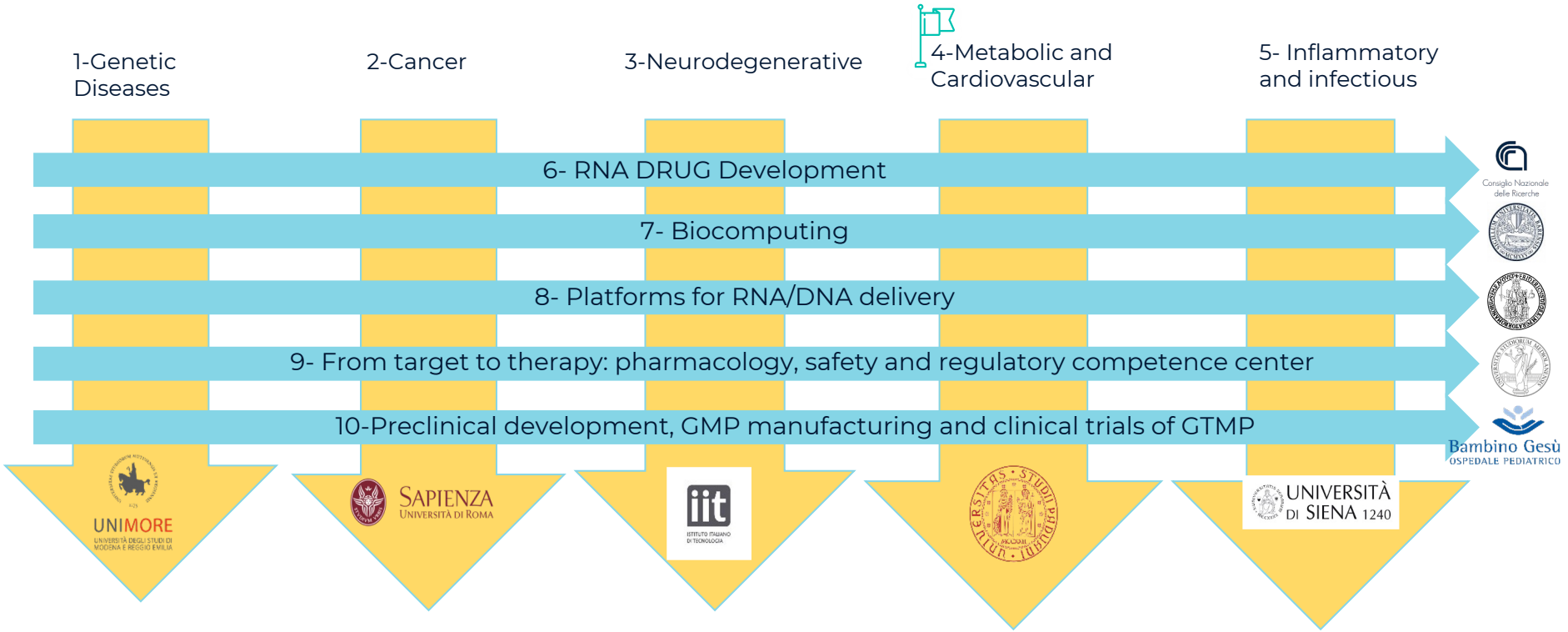
▶ HORIONTAL SPOKES

Development of RNA/DNA drugs
Biocomputing
Platforms for RNA/DNA delivery
Pharmacology, safety and efficiency studies
Preclinical development, GMP manufacturing and clinical trials of GTMP

To reach the objectives of the Center 10 thematic areas were identified (spokes). Vertical spokes refer to diseases of high impact for which the NC proposes new therapies. Horizontal spokes aim to develop the KET and produce high quality products in large scale.

National Center for Gene Therapy and RNA-based Drugs

The Coordinators of the Spokes



PUBLIC AND PRIVATE FOUNDERS

Public Universities and Research Entities

Università di Modena e Reggio Emilia*
Università di Napoli *
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Università di Padova *
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Foundations- IRCCS- Private Universities

IIT*
Ospedale pediatrico Bambino Gesù*
Humanitas-Università
Ri.MED
Telethon (Tigem, Tiget)
Università Vita Salute San Raffaele
Fondazione Tettamanti

Private Enterprises

Orgenesis
Chiesi
Novartis
Pfizer
Biontech
Sanofi
CDI
Astrazeneca
Antares
IRBM
Takis
PBL
Innovavector
Stevanato
Intesa San Paolo

The group leaders include 6 top scientists with a Scopus h-index >100 (Lista, Priori, Locatelli, Pelicci, Rizzuto, Naldini), 6 with an h- index >90 (Zeviani, Biondi, Ballabio, Di Fiore, Sozzani, Zinzani), and overall >100 senior investigators with an h-index >50, >200 with h-index >40. 17 participants are EMBO members (Bozzoni, Ciliberto, D'Adda Di Fagagna, De Luca, De Matteis, Del Sal, Hirsch, Matteoli, Mavilio, Naldini, Pasini, Pelicci, Piccolo, Poli, Rizzuto, Scita, Scorrano).

THE COMPANIES IN SPOKES

BUDGET BREAKDOWN (320 M€)

Research activity (199 M€)
- Research grants (TRL development grants)
- Non-tenured faculty positions

Training (14 M€)
- Ph.D. programs of affiliated entities
- National Ph.D. program (Gene Therapy and RNA-based Drugs) and Life-long Learning initiatives

Infrastructures (87 M€)
- RNA platform facility (to be built in Naples)
- Implementation of the Gene Therapy Consortium (Bambino Gesù Hospital, TIGET, Fondazione Tettamanti)
- Spoke flagship

Technology Transfer (17 M€)
Spin-off and start-up accelerators

Management (3 ME)