



# RNA & GENETHERAPY

CENTRO NAZIONALE DI RICERCA



Finanziato  
dall'Unione europea  
NextGenerationEU



Ministero  
dell'Università  
e della Ricerca



Italiadomani  
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DI RIPRESA E RESILIENZA

RNA   
& GENETHERAPY



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CENTRO NAZIONALE DI RICERCA



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# About the National Center

## What is the National Center for RNA & Gene Therapy?

The National Center for Gene Therapy and Drugs based on RNA Technology, funded by the EU National Recovery and Resilience Plan NRRP Mission 4 – Component 2, Investment 1.4, and spearheaded by the University of Padua, brings together 46 Italian and international partners from the public, private, and corporate sectors and organizes them within a 36-month research program.

Activities of the research program aim to field a better understanding of diseased molecular mechanisms and to develop therapies for them. Its overarching strategic goals are to support the efficacy and sustainability of the healthcare system, within the framework of a competitive knowledge-based economy.

The National Center is home to many research projects structured along the Hub and Spoke organizational model, which allows for the interconnection of Vertical Spokes and Horizontal Spokes.

## What is RNA and gene therapy?

Compared to traditional therapies, RNA offers direct action on specific molecular targets and flexible administration, which can vary from once a month to once every six months. Looking ahead, RNA-based therapies could dominate the medical field, leveraging years of genetic studies by selectively targeting the cause of diseases.

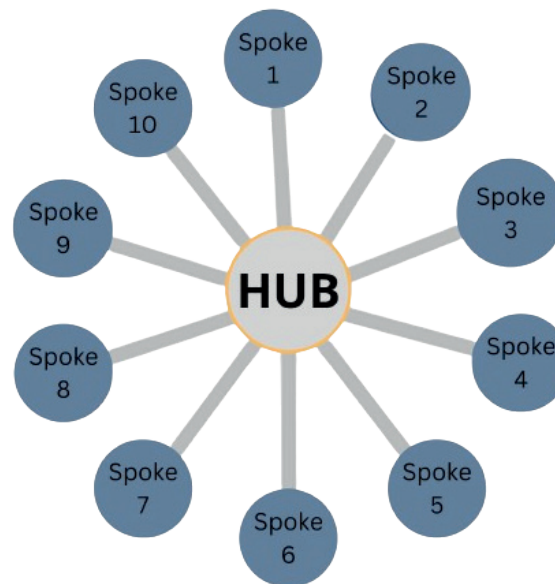
Gene therapy represents a revolutionary breakthrough that offers innovative and effective treatments in the field of medicine. Gene therapy eliminates the need for ongoing treatments and reduces patient care costs, representing a sustainable investment in the future of healthcare.



# Spokes

Structured along a Hub and Spoke dissemination model, the National Center Foundation is the Hub facilitating the interconnection between 10 Spokes that aim to translate research results into the development and manufacturing of personalized medicines.

Vertical Spokes focus on identifying the most promising candidate targets for RNA-based drugs, while Horizontal Spokes aim to build and disseminate the technology to design, deliver, and produce gene therapy products and RNA drugs. The National Center Foundation works to interconnect Spokes toward expanding RNA-based pharmacology and gene therapy and developing new drugs for precision medicine.



**SPOKE 1 | Genetic Diseases**

University of Modena and Reggio Emilia

**SPOKE 2 | Cancer**

Sapienza University of Rome

**SPOKE 3 | Neurodegeneration**

Italian Institute of Technology

**SPOKE 4 | Metabolic and Cardiovascular Diseases**

University of Padua

**SPOKE 5 | Inflammatory and Infectious Diseases**

University of Siena

**SPOKE 6 | RNA Drug Development**

National Research Council of Italy

**SPOKE 7 | Biocomputing**

University of Bari Aldo Moro

**SPOKE 8 | Platforms for RNA/DNA Delivery**

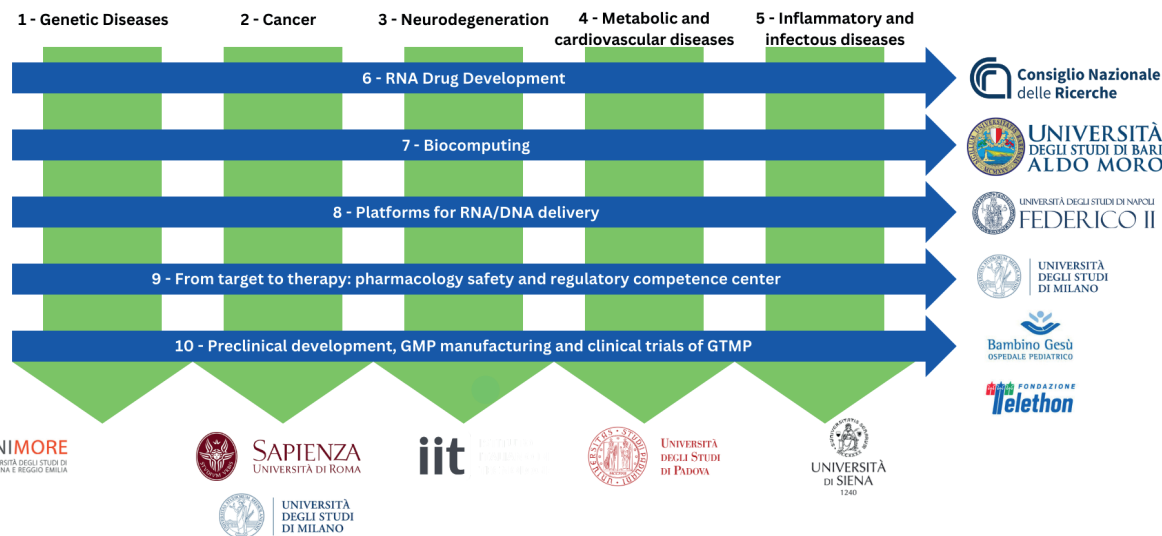
University of Naples Federico II

**SPOKE 9 | Competence Center on RNA Drug Pharmacology**

University of Milan

**SPOKE 10 | Pre-clinical Development, GMP Manufacturing and Clinical Trials of GTMP**

IRCCS Bambino Gesù Children's Hospital in Rome



# Mission and Members

## MISSION

Guided by science and powered by technology, the National Center is committed to delivering RNA-based solutions from the lab to patients. With an understanding and the competence to deliver breakthrough medicine to the market, the National Center aims to make healthcare more effective, efficient, and sustainable while fostering education, investing in step-up facilities, and cultivating a dialogue between those involved in the discovery and those implementing gene therapy and drugs based on RNA technologies.



## MEMBERS



# Return on Investments

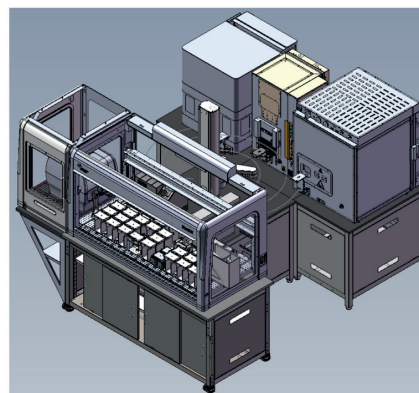
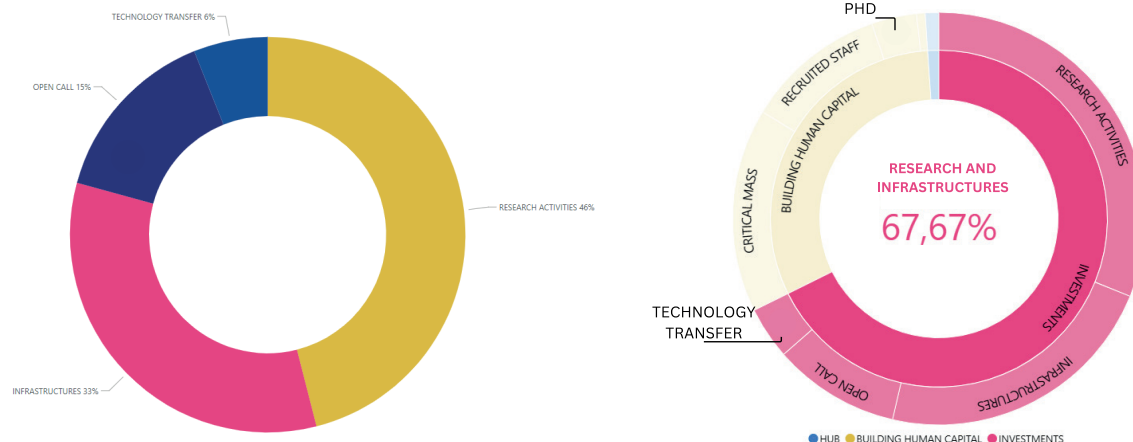
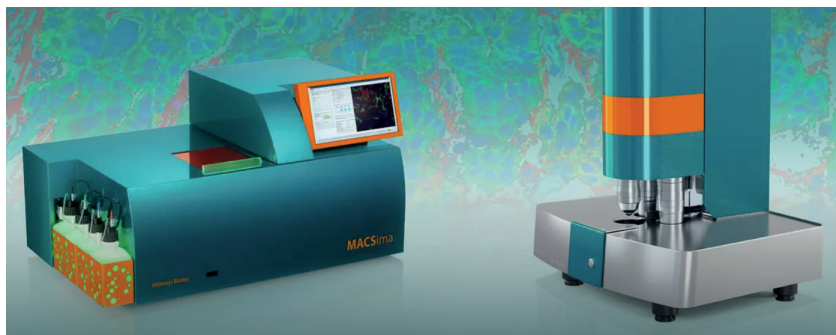
Advancing towards an innovative moment in RNA-based therapeutics, the strength of the National Center lies in the various research activities, infrastructures, open calls, and technological transfer needed to bring pharmaceutical products to the marketplace.

Research activities of the National Center cover human diseases related to genetics, cancer, metabolic and cardiovascular diseases, neurodegeneration, and inflammatory and infectious disorders. Other activities aim to translate research into tangible products through biocomputing, RNA/DNA delivery platforms, pre-clinical development, and regulatory experts that design scalable protocols for industrial production.

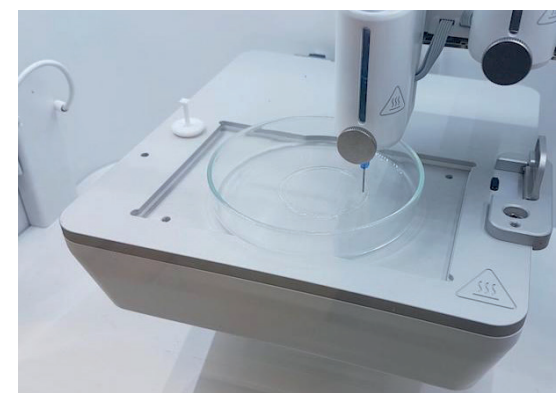
The National Center promotes enhancing current facilities and developing infrastructures for the study, development, and manufacturing of final products according to national and international regulations.

Open Calls aims to bring forth new skills outside the current scope of the National Center and its relative affiliates by expanding the research perimeter and exceeding its technological profile.

By connecting stakeholders and moving scientific findings from the laboratory to public and private uses, the technological transfer of the National Center aims to benefit society as a whole. Following intellectual property standards and patent processes, the Center engages in the commercialization and marketing of gene therapy and drugs based on RNA technology, while supporting the success of spin-offs to further improve Europe's economic competitiveness.



Robotic platform for automation of cellular assays



Bioprinter 3D

Platform for 2D/3D imaging of complex biological samples by light-sheet microscopy coupled with spatial multi-omics analysis of RNA and proteins



# Infrastructures

The National Center research program promotes the creation of state-of-the-art infrastructures and spoke flagships that are accessible to all its members and the broader scientific community.

The Gene Therapy Center will take advantage of pre-existing facilities located at Bambino Gesù Children's Hospital in Rome (OPBG), the San Raffaele Telethon Institute for Gene Therapy (TIGET-SR) and the Tettamanti Foundation. Determined to fulfill its objectives, the Gene Therapy Center focuses on improving the clinical translation of Gene Therapy (GT) products and creating a service structure for the production of GT products.

The RNA Production Platform will occupy a dedicated space at the University of Naples Federico II and will serve National Center research groups as a space devoted to RNA synthesis and formulation. Equipped with advanced instrumentation, the facilities offer a RNA Production Platform capable of making the manufacturing processes agile, robust, and reproducible.

Spoke Flagships represent a significant leap forward in our ability to understand, treat, and ultimately cure rare, severe, and often incurable disorders. By improving and developing laboratories with the latest cell culture technology, imaging systems, molecular biology tools, and more, Spoke Flagships will foster groundbreaking research. By expanding research infrastructures to meet the needs of its ambitious program, Spoke Flagships will reshape delivery strategies while ensuring that innovative drug products are ready for early-stage clinical trials.



Gene Therapy Center wet lab space

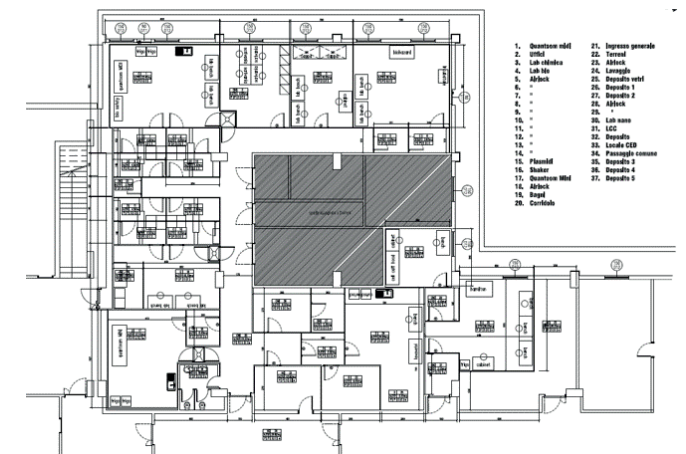
One of many National Center funded laboratories



RNA Production Platform

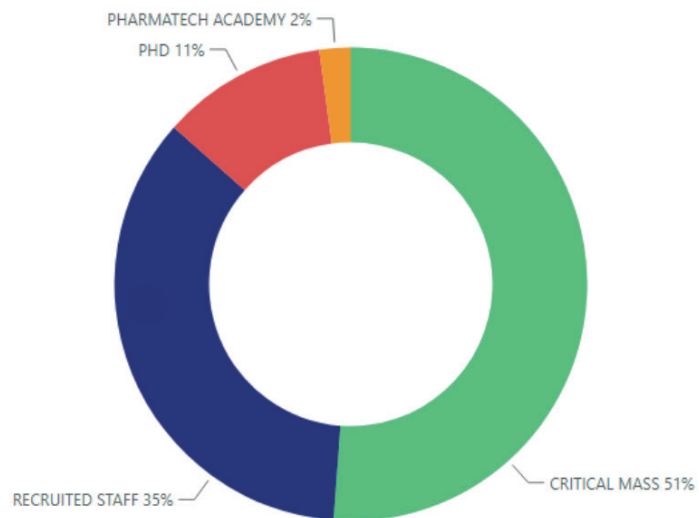
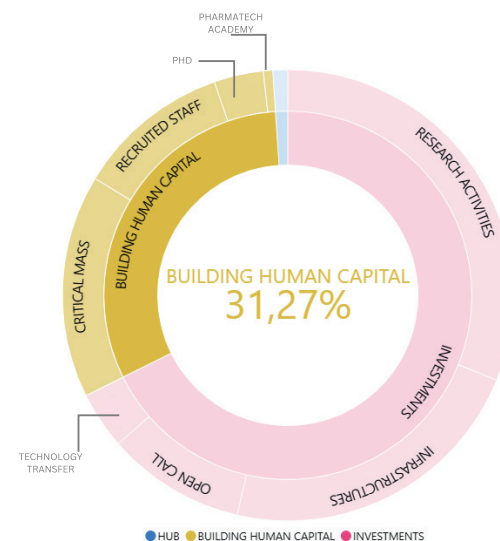


RNA Production Platform Blueprint



# Building Human Capital

The success of the National Center relies on its ability to bring together those with various skill sets, competencies, and talents. Connecting collaborative relationships with other research centers, universities, companies, and new business ventures, demonstrates the National Center's commitment to Building Human Capital. The National Center has so far recruited about 500 researchers, including more than 170 PhD students and 330 RTD researchers, technologists, and research fellows. Encouraging creativity, innovation, and a lifelong learning-based culture, the National Center fosters a workplace that is diverse, equitable, and inclusive.



# National PhD and PharmaTech Academy

## National PhD

The RNA Therapeutics and Gene Therapy National PhD program aims to train highly qualified researchers with the scientific expertise and autonomous research skills needed to address the challenges of RNA-based drugs and gene therapy.

As a comprehensive three-year doctoral degree program, guided by the Department of Pharmacy at the University of Naples Federico II, the program involves ten universities and two research centers across Italy.

## PharmaTech Academy

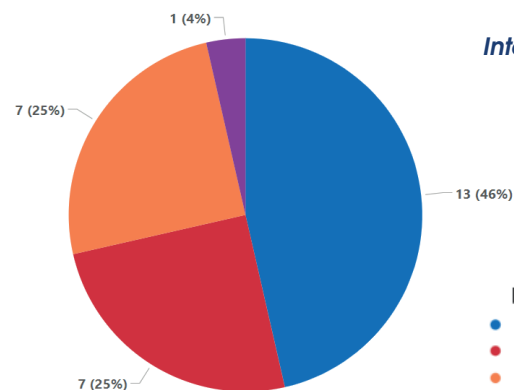
PharmaTech Academy is a training initiative of the National Center led by the University of Napoli Federico II, Department of Pharmacy. Its main objective is to train highly qualified professionals to operate across all phases of RNA-based drug development, thanks to a practical and professional educational approach based on the concept of learning by doing.

Built on a close collaboration between universities and private companies participating in the National Center, the Academy aims to expand the skills acquired during degree programs by offering hands-on experience and direct contact with the pharmaceutical industry, thus contributing to the creation of specialized figures in the field of RNA gene therapy in Italy.



National Center for Gene Therapy and Drugs based on RNA Technology

## Interdisciplinary Background - Class of 2024



### Master Degree

- Biology
- Medical, Pharmaceutical, and Veterinary Biotechnologies
- Pharmacy and Pharmaceutical Sciences
- Chemical sciences



## Communication

### Magazine - The Biopharma Encapsulated Series

The National Center BioPharma Encapsulated magazine series aims at making science and innovation accessible to a wide audience. The latest series features insight into what the National Center does and details each of its 10 Spokes. The magazine is a dissemination tool for the understanding of subjects that may otherwise seem complex for non-scientists to comprehend.

"We have the responsibility to extend the benefits of our research to all patients, especially those suffering from rare diseases. The Spoke 1 research project foresees joint research and development paths with the business world to ensure that scientific discoveries become real therapeutic solutions." Says Prof. Antonello Pietrangelo, Spoke 1 scientific coordinator .

### News

Highlighting recent publications from National Center researchers is a vital part of public engagement. From streamlining protocols towards precision medicine to identifying biomarkers for personalized treatment, sharing published results is how the National Center ensures that a wide audience has access to the latest improvements in the diagnosis, management, and treatments of disease.

### LinkedIn

Social media is a major part of daily life. It allows us to stay up-to-date on current events and offers a way for us to communicate with those near and far. The National Center's LinkedIn page shares the latest in research activities, open calls, and networking opportunities. Creating an online presence is synonymous with forging strategic partnerships and future collaborations.

### YouTube

The dedicated National Center YouTube page aims to put faces to the names of all those involved in its innovative program. From highlighting events with important public figures to giving a voice to young researchers, YouTube allows us to share content to enhance the National Center's goals.



## Events

Participating and organizing private and public events allows the National Center to efficiently communicate its mission and goals.

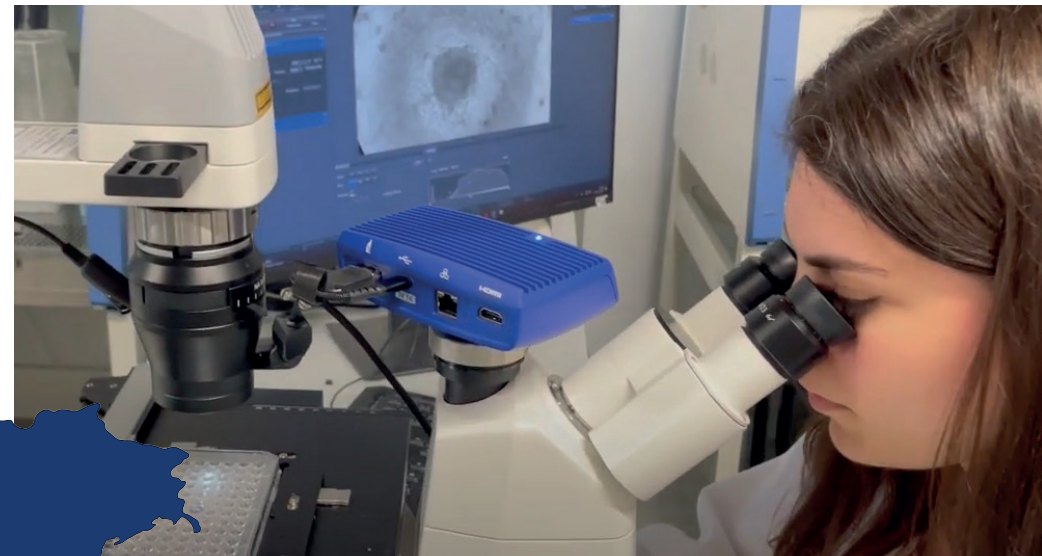
On Monday 20 November 2023, the National Center outlined its strategic goals and research projects to the public. Hosted by the University of Padua, the event welcomed governing members of the National Center along with representatives from several Italian institutions, distinguished scholars, and the participation of Italy's Minister of University and Research Anna Maria Bernini.

On Saturday 23 March 2023, joined alongside an international panel of speakers, the President of the National Center, Prof Rizzuto participated in the World Health Forum. Acknowledging that when considering the idea of a cure we often perceive rare diseases as orphans, Prof Rizzuto reminds us that all diseases can be broken down into subgroups, and thus treated through personalized medicine. Knowing the enormous cost of developing specific targets lies in refining the delivery methods of medicines through a common platform, Rizzuto confirms that this is where the National Center excels. By developing drugs based on RNA technology, the National Center exploits various types of RNA that can make, modify, inhibit, or change the activities of proteins within the genome path.



## Open Calls

Aimed at facilitating and expanding research activities, the National Center for Gene Therapy and Drugs based on RNA Technology activates Open Calls proposed by Spokes. Open Calls focus on acquiring new skills and technologies outside the current scope of National Center Spokes and relative affiliates. Funds distributed in Open Calls serve to expand the research perimeter and the competence portfolio needed to bring research results to fruition, thus expanding the technological expertise and accelerating the process of designing and developing new treatments.



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Fondazione Centro Nazionale di Ricerca - Sviluppo di Terapia Genica e Farmaci con Tecnologia a RNA

**Legal Address:** via 8 Febbraio, 2 – 35122 Padua, Italy - **E-mail:** [ricerca@RNA-genetherapy.eu](mailto:ricerca@RNA-genetherapy.eu) - **Certified email:** [terapiagenica-farmacirna@legalmail.it](mailto:terapiagenica-farmacirna@legalmail.it) **Tax ID code (codice fiscale)** IT92315700283

NRRP Next Generation EU – Mission 4, Component 2, Investment 1.4 “National Center for Gene Therapy and Drugs based on RNA Technology” - CUP HUB B93D21010860004