



**Fondazione Ricerca  
Fibrosi Cistica - ETS**

*italian cystic fibrosis research foundation*

# **FFC RICERCA: AN OVERVIEW OF SCIENTIFIC ACTIVITIES**

Mission and key  
achievements to  
enhance therapies and  
improve the quality of  
life for people with cystic  
fibrosis.

**A CURE FOR ALL**

# IDENTITY

The Foundation is a non-profit body recognised by the Italian Ministry of University and Research (MUR) as a promoter of scientific research on cystic fibrosis. It was established in 1997 in order to:



**Promote and fund**  
scientific research of special  
social interest on cystic  
fibrosis (CF)



**Train**  
researchers and health  
professionals working  
with CF



**Contribute to the  
management of  
research laboratories**  
with specific agreements



**Spread information**  
about cystic fibrosis  
among the general public

## CYSTIC FIBROSIS IN ITALY

It is one of the most common genetic diseases and there is still no definitive cure. The disease is present since birth and is caused by mutations in the CFTR gene that make the bodily fluids more dense, deteriorating the function of lungs, pancreas and other organs. The extent of the lung damage determines the quality of life and the longevity of people with CF.



People with CF have inherited a mutated gene from both their father and mother who are, in most cases, unaware of being carriers of a mutated CFTR gene.

### CF PATIENTS

**6,000**  
estimated  
people

**2**  
newborns  
with CF every  
week

**1**  
child  
with CF  
in 3,000  
births/year

**2,000**  
known  
mutations  
of the CFTR  
gene

### CARRIERS

**2**  
million  
healthy  
carriers in Italy

**1**  
person  
in 30

**1**  
couple  
in 900

At each pregnancy, the newborn

**25%**

has  
FC

**25%**

is healthy,  
not carrier

**50%**

is a healthy  
CF carrier

**★ Goal 3 “Good health and well-being for all”**

is our primary contribution to the 2030  
Agenda for sustainable development.

**3 GOOD HEALTH  
AND WELL-BEING**



# MISSION

These activities cover the goals that the Foundation was established for.....



## HIGHLIGHTS, A SELECTION OF PROGRESS MADE IN 2024



Selected **Publications** in international scientific journals with high impact factor supported by FFC Ricerca projects

### \* Evolution and host-specific adaptation of *Pseudomonas aeruginosa*

Science. 2024 Jul 5;385(6704): eadi0908.  
Project: FFC#10/2023  
Investigator: Giordano Rampioni (Roma Tre University)

### \* A functional 3D full-thickness model for comprehending the interaction between airway epithelium and connective tissue in cystic fibrosis

Biomaterials. 2024 Jul;308:122546.  
Project: FFC#14/2019  
Investigator: Paolo Netti (Istituto Italiano di Tecnologia-IIT - Naples; University of Naples, Federico II)

### \* Single-Cell RNA Sequencing Shows that Circulating Monocytes Enriched in IFN Signaling Are Associated with Nontuberculous Mycobacteria Pulmonary Disease in Cystic Fibrosis

Am J Respir Crit Care Med. 2024 Sep 15;210(6):834-837.  
Projects: FFC#7/2022 and FFC#23/2020  
Investigator: Nicola Ivan Lorè (San Raffaele Hospital, Milan)

### \* Prime editing functionally corrects cystic fibrosis-causing CFTR mutations in human organoids and airway epithelial cells

Cell Rep Med. 2024 May 21;5(5):101544.  
Project: FFC#2/2021  
Investigator: Anna Cereseto (CIBIO, University of Trento)

### \* Tezacaftor is a direct inhibitor of sphingolipid delta-4 desaturase enzyme (DEGS)

J Cyst Fibros. 2024 Nov;23(6):1167-1172.  
Project: FFC#1/2023  
Investigator: Andrea Armirotti (Istituto Italiano di Tecnologia-IIT, Genoa)



Applications and achievements of patents and **ODD** from studies funded by the Foundation

28 on our projects

10 of which are co-owned by FFC Ricerca

1 in the process of acquiring co-ownership

1 Orphan Drug Designation (ODD)



Strategic projects with direct impact on the lives of people with CF, their families and the general population

### \* Kaftrio in the real life

A study to assess the efficacy and safety of Kaftrio

### \* 1 in 30 and you don't know it

A campaign for a better comprehension and awareness of the CF carrier test

### \* Experts together

An educational project on the basis of clinical research addressed to a group of people with CF and their parents

# MISSION ACTIVITIES

## SCIENTIFIC RESEARCH

The Foundation selects and funds innovative studies on cystic fibrosis with the aim of:

### Developing new therapies to counter:

- ✳ the effects of CFTR mutations, even the rare ones
- ✳ bacterial and fungal infections of the airways
- ✳ pulmonary inflammation

### Evaluating the long-term effects of CFTR modulators

### Increasing the knowledge about the disease

With the following tools:



2

calls for proposal  
(standard + for young researchers)



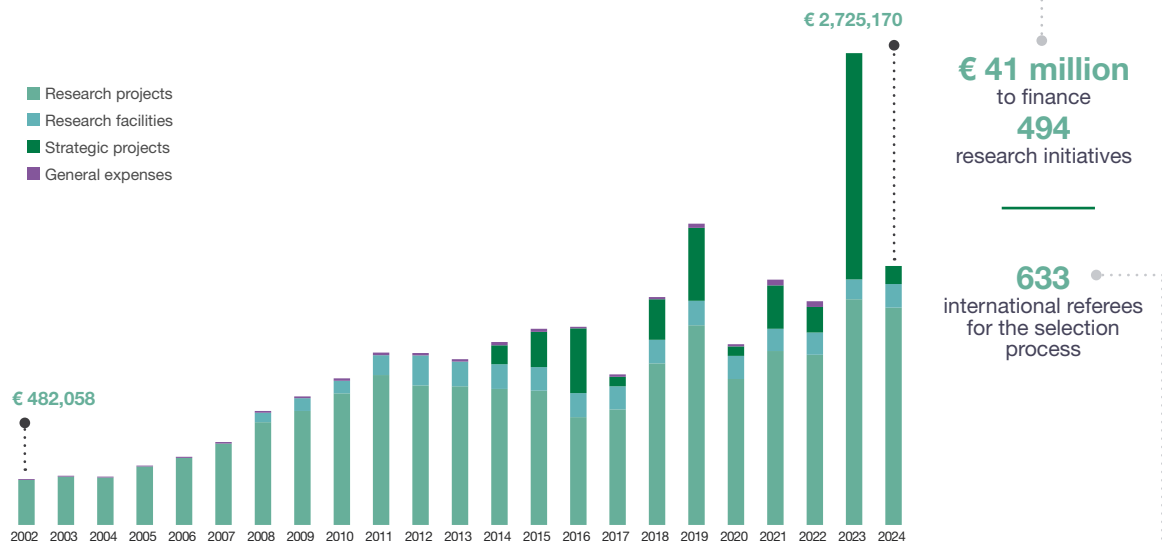
3

research facilities  
(CFaCore, SCP, CFDB)



3

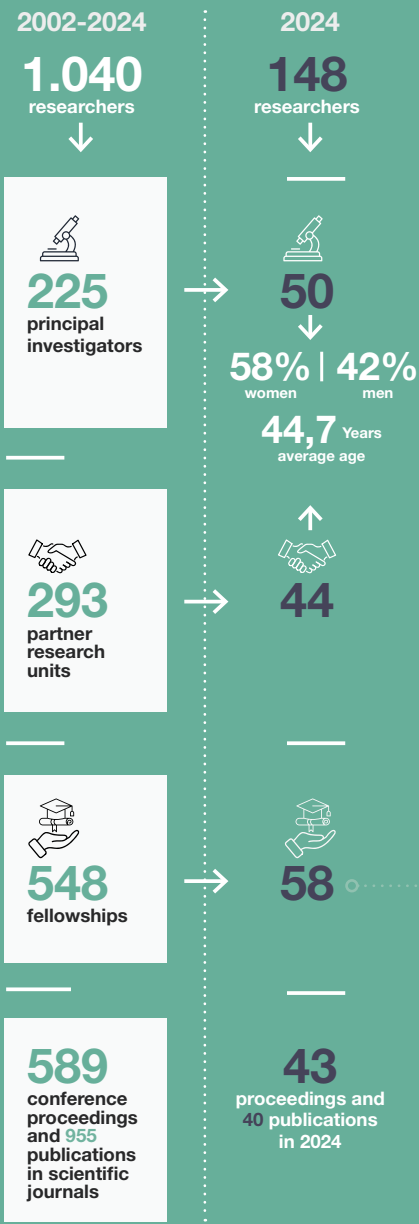
strategic projects  
(GenDel-CF, Kaftrio Effect, Molecules 3.0)



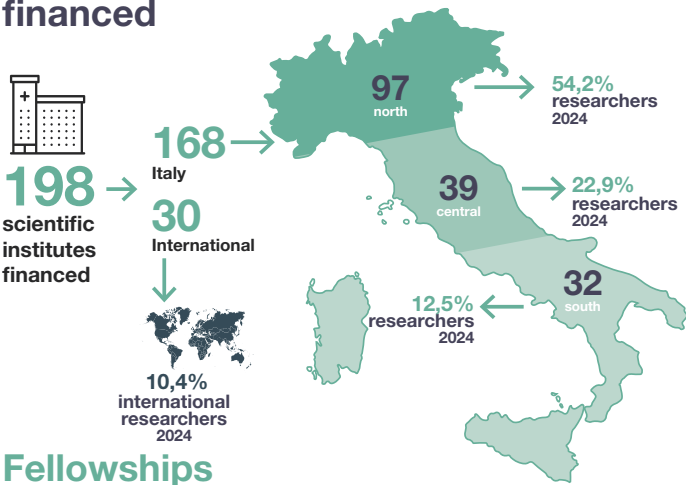
## THE SELECTION OF PROJECTS PROPOSAL



# FFC RICERCA SCIENTISTS NETWORK



## 2002-2024 Scientific institutions financed

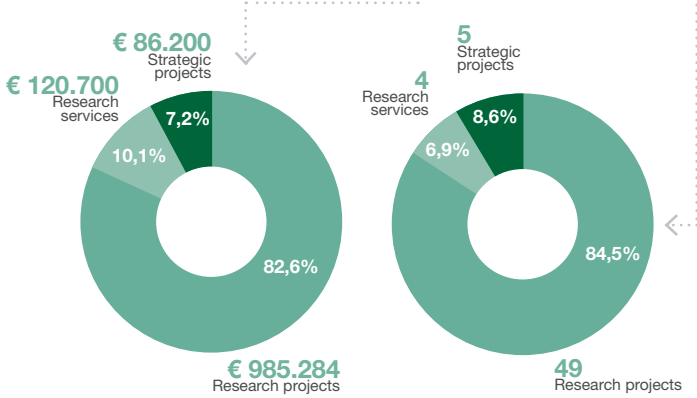


## ➤ Fellowships

2002-2024



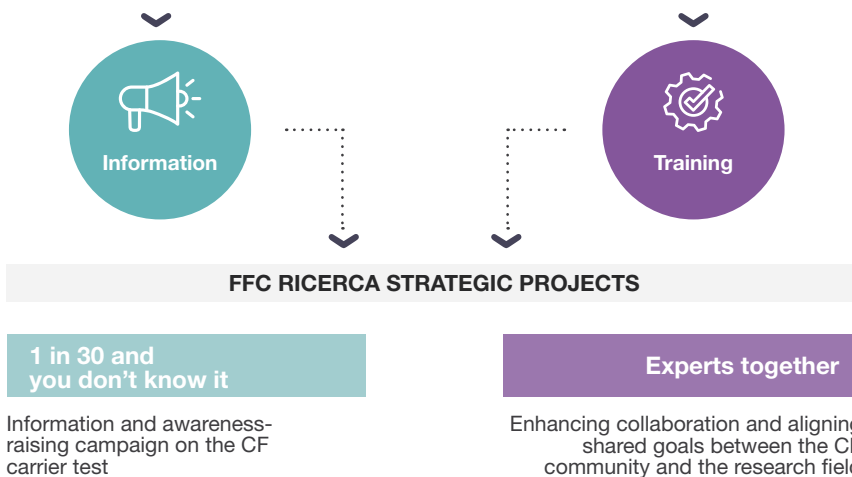
2024



## MISSION ACTIVITIES

# INFORMATION AND TRAINING

Strategic projects and awareness-raising initiatives that aim at informing and training. These comprise



## 2024 INITIATIVES AND MEETINGS

### Transparent Research



**69**

sponsors received the results of the research they funded

### Convention of Investigators

**250**

participants



### CF community Seminar



**250**

participants



### CONVENTION OF FFC RICERCA INVESTIGATORS

For over 20 years the FFC Ricerca Foundation, in mid-November, has been organizing a three-days meeting of the research network financed. The event is addressed to medical doctors, researchers and experts in the CF field. In last years the official language of the meeting switched from Italian to English with also the opportunity to follow the event online streaming.

Highlights of the event are available at



# GOVERNANCE

The Foundation relies on government and supervisory bodies to ensure efficient procedures, verify transparency and guarantee compliance with its statutory mission.

## BOARD OF DIRECTORS

It defines the general directives, approves the financial statements and social report.

*President*  
**Matteo Marzotto**



## THE SCIENTIFIC ADVISORY COMMITTEE

It selects the best projects in cooperation with the Scientific Direction

*President*  
**Paolo Bernardi**



## THE SCIENTIFIC DIRECTION

It establishes research strategies in collaboration with the Scientific Advisory Committee.

*Director*  
**Carlo Castellani**  
*Deputy-Director*  
**Nicoletta Pedemonte**



# THE VOLUNTEERS NETWORK

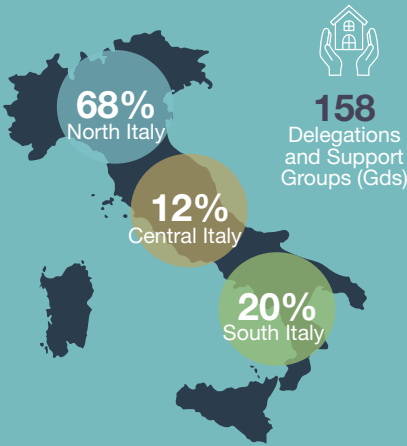
The FFC Ricerca collaborates with a broad network of volunteers in order to



Inform on the disease



Raise funds for research by organising campaigns and events

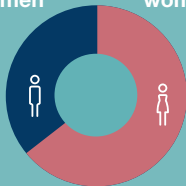


950

regular volunteers registered in the related Register (as on 31/12/2024)

↓27% men

↑73% women



49 Years average age



2024

€ 3,241,205 raised by volunteers for the projects selected through the 2024 calls for proposal



*Fondazione per la Ricerca  
sulla Fibrosi Cistica - ETS  
italian cystic fibrosis research foundation*

c/o Azienda Ospedaliera Universitaria Integrata  
Piazzale Stefani 1  
I-37126 Verona  
Tel. +39 045 8123438  
CF 93100600233

[fondazione.ricercaffc@aovr.veneto.it](mailto:fondazione.ricercaffc@aovr.veneto.it)

For further information



[www.fibrosicisticaricerca.com](http://www.fibrosicisticaricerca.com)

## **TO DONATE TO THE FONDAZIONE RICERCA FIBROSI CISTICA**

- Online on the website: [dona.fibrosicisticaricerca.it](http://dona.fibrosicisticaricerca.it)
- UniCredit Bank transfer (no commission at UniCredit Bank branches)  
IT 47 A 02008 11718 000102065518  
BIC SWIFT code (for payments from abroad): UNCRITM1N58
- Banco BPM: IT 92 H 05034 11708 0000000048829  
BIC SWIFT code: BAPPIT21008
- Postal account nr.: 18841379
- 5x1000 (0.5% income tax credit scheme) tax code: 93100600233



DONARE CON FIDUCIA

FFC Ricerca is recognised by the Italian Institute of Donation, which certifies the transparent and effective use of funds raised, to protect the rights of donors.

Follow us on

