



Fondazione per la Ricerca
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**ITALIAN CYSTIC FIBROSIS RESEARCH FOUNDATION (FFC Ricerca)
“GIANNI MASTELLA” CALL FOR APPLICATIONS
“GIANNI MASTELLA STARTING GRANT”
AND
“GIANNI MASTELLA RESEARCH FELLOWSHIP”
YEAR 2024**

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1. Introduction and features of the FFC Ricerca 2024 “Gianni Mastella” call

The Italian Cystic Fibrosis Research Foundation (FFC Ricerca) funds research projects that have the ultimate aim to improve the health status of persons with cystic fibrosis (CF) and to providing a breakthrough in understanding of the molecular basis of the disease.

This call is in the memory of prof. Gianni Mastella, pediatrician and key scientist in CF research and co-founder of FFC Ricerca. Two funding options are available:

- the “**Gianni Mastella Starting Grant - GMSG**” is addressed at investigators operating in the field of CF to continue their scientific career in the study of the basic defect of the disease or in the development of therapeutic approaches to improve the life of people with CF.
- the “**Gianni Mastella Research Fellowship - GMRF**” is addressed at young researchers who want to start their career in CF research in a laboratory already active in the field.

FFC Ricerca anticipates to finance 1 GMSG and 1 GMRF, depending on the type and quality of the applications and the availability of funds.

Relevant features of this call

- **Gianni Mastella Starting Grant – GMSG:**
 - the maximum budget allowed is **180.000 euros for the entire project**;
 - the budget will cover:
 - the cost of the **PI’s salary**, set at **31.000 euros** maximum per year for a total of 93.000 euros for the entire period,
 - the cost to conduct the **research activity** set at a maximum of 87.000 euros for the entire period.
 - the candidate must be **40 years old or younger** (see Eligibility criteria below);
 - the PhD or MD degree is mandatory;
 - the candidate must have **at least 3 publications** as first/last/corresponding author in original papers published in international peer-reviewed journals;
 - the applicant must provide a **recommendation letter** by the chief of the lab in which his/her **mentorship** to the applicant is assured;
- **Gianni Mastella Research Fellowship – GMRF:**
 - the candidate must be **33 years old or younger** (see Eligibility criteria below);
 - the maximum budget allowed is **100.000 euros for the entire project**;
 - the budget will cover:
 - the cost of the **research fellowship**, set at 25.000 euros per year for a total of 75.000 euros for the entire period,
 - the cost to conduct the **research activity** set at 25.000 euros for the entire period.
 - the PhD or MD degree is NOT mandatory;
 - the candidate must have **at least 1 publication** in the field of cystic fibrosis,
 - the applicant must provide a **recommendation letter** by the chief of the lab in which his/her **tutorship** to the applicant is assured;
- **Common features:**
 - deadline for submitting applications: **31st January 2024**;

- only **monocentric projects** are allowed;
- the duration of the projects is set at **36 months**;
- a **training period** in an External Research Institution (ERI) is encouraged (see Project duration and training period below);
- the candidate must produce a document certifying the willingness of the host institution and, if applicable, of the external research center to conduct the research;
- the funding grant starts on **1 September 2024**.

2. Priority areas

The GMSG and GMRF Call for grant applications aims at funding projects focused on the following 5 priority areas in the field of CF research:

2.1. Understanding and treating CFTR basic defects

Design of new approaches to correct defective CFTR or to compensate for its deficient function with the following particular indications:

- better understanding of CF pathophysiology;
- novel mutation-specific therapies;
- ancillary supports to modulator therapy;
- targeting alternative chloride channels;
- development of therapies based on gene or RNA editing.

2.2. Personalized therapies

Identification and validation of new and appropriate *in vivo* and *ex vivo* models and assays, such as *therotyping*, to predict and monitor the potential efficacy of new therapies finalized to correct the CFTR defect.

2.3. Airway infection in CF

Development of innovative diagnostic and antimicrobial strategies with special indication for the rapid and accurate diagnosis of infections and the treatment of difficult-to-treat microorganisms.

2.4. Inflammation in CF

Innovative strategies to contain the inflammation-based pathology.

2.5. Clinical applications and epidemiological studies

- Clinical trials, with special regard to:
 - phase IV clinical studies (post-marketing studies in the real life);
 - studies to improve the outcomes of lung transplantation in CF;
 - studies of efficacy (randomized and controlled phase II/III) are not financed by this call, but the FFC Ricerca can contribute by partially co-funding the study. FFC Ricerca reserves the right to discuss on a case-by-case basis.
 - phase I/II studies in which biomarkers of efficacy/safety are also tested;
 - innovative diagnostic approaches to predict and monitor the evolution of the disease also in the context of new therapies;
- Epidemiological studies, with special regard to:
 - studies on data collected from the Italian CF Registry database;
 - systematic reviews and meta-analyses (in particular network meta-analyses);
 - updates of traditional therapies that involve a heavy burden on sick people;
 - studies on the correlation between climate changes, environment and the health of persons with CF.

3. General notes and recommendations

- Research proposals in which **translational objectives** are clearly evident will be prioritized.
- Accordingly, research projects dealing with either **clinical studies or pre-clinical studies** exploiting animal models of CF or performed on primary CF cells culture are encouraged.
- Projects involving **international collaborators** are encouraged.
- FFC Ricerca also encourages projects focused on studies on **rare mutations and/or mutations not susceptible to current available modulators**.
- *In vitro* studies dealing with non-CF cell lines are strongly discouraged, unless required for preliminary experiments or used to reinforce data obtained with primary CF cells.
- The study must be supported by preliminary data already acquired; **this call does not fund proof of concepts (PoC)**.
- Researchers should consider the **facilities offered by FFC Ricerca** in the design of the projects presented (see also Appendix 1):
 - **Servizio Colture Primarie (SCP)** which collects primary cell cultures obtained from bronchial epithelium of CF and non-CF patients. For further details please visit the dedicated [webpage](#) and contact the service coordinator.
 - **Cystic Fibrosis animal Core Facility (CFaCore)** which develop and provides mouse models for research applications in CF field. For further details please visit the dedicated [webpage](#) and contact the service coordinator.
 - **Cystic Fibrosis Data Base (CFDB)**, the data base of clinical interventions in CF. For further details please visit the dedicated [webpage](#) and write at info@cfdbeu.
- **FFC Ricerca facilities** (SCP, CFaCore, CFDB) must be considered as a service, therefore the facility coordinators must not be indicated as partners or collaborators. Please see guidelines at point 4 (Outside Collaborations/Services) to fill in the Form 9.
- FFC Ricerca encourages **repurposing** studies if there is a strong rationale that demonstrates the validity of the approach in the context of CF pathology.
- Studies aimed at identifying new therapeutic compounds will also be taken into consideration, provided that they suggest new strategies to affect mutated CFTR function and/or CFTR-dependent mechanisms of cell pathology, also including modification of DNA and RNA. Both types of studies may exploit *in vitro* or *ex vivo* primary cell models.
- FFC Ricerca will keep funding *in vitro* and *in vivo* studies addressing effects of drug/compounds not yet tested in CF. The project has to aim at assessing the degree of efficacy and toxicity in CF, possibly addressing the molecular mechanisms of efficacy. Due to funding limitations, FFC Ricerca will not fund "Good laboratory practice" (GLP) compliant preclinical studies for the development of a compound already tested in preclinical studies or to apply for the permission to start a clinical trial. The study can only be partially outsourced to companies in order to collect preliminary data relevant to submit a request of Patent or alternatively Orphan Drug Designation of the molecule under study.
- Studies aimed at identifying **new antimicrobial strategies** will only be considered if original strategies are proposed, along with enough preliminary data, to support a potential advantage vs. conventional treatment protocols. FFC Ricerca will not consider studies aimed at the identification or preliminary characterization of hit compounds active against multidrug-resistant pathogens unless a clear advantage over current agents (even at the pre-clinical stage) is expected for treating CF infections.

- Collaboration and transferring of knowledge and expertise from basic to clinical research is particularly recommended. To this aim the advice of a clinical consultant for basic research projects is conceivable and her/his role must be clearly highlighted in the application cover letter.
- By "translational research" this call means not only "bench-to-bedside" studies. Research projects considering the translation of results from clinical studies into everyday clinical practice and health decision making are also welcome. Topics to be considered include clinical epidemiology, communication, behavioral science, organizational theory, quality monitoring and quality improvement research.
- The laboratories that will be financed will have to provide the necessary documentation relating to the correct disposal of laboratory waste, as required by D.M. 4/7/2019 (*Adozione delle Linee guida per la redazione del bilancio sociale degli enti del Terzo settore*), by the end of the projects. Further information will be provided to entities deserving of funding.

4. Eligibility criteria

- Eligible applicant
 - **Gianni Mastella Starting Grant – GMSG:**
 - at most 40 years old (born after 31 August 1984); exceptions might be granted on a case-by-case basis for duly justified reasons, for example for maternity, paternity or serious illness;
 - not having a permanent position in the host institution (see Budget for details);
 - the PhD or MD degree is mandatory;
 - the candidate must have at least 3 publications as first/last/corresponding author in original papers published in international peer-reviewed journals;
 - a recommendation letter by his/her mentor is mandatory.
 - **Gianni Mastella Research Fellowship – GMRF:**
 - at most 33 years old (born after 31 August 1991); exceptions might be granted on a case-by-case basis for duly justified reasons, for example for maternity, paternity or serious illness;
 - not having a permanent position in the host institution (see Budget for details);
 - the PhD or MD degree is NOT mandatory
 - the candidate must have at least 1 publication in the field of cystic fibrosis,
 - a recommendation letter by his/her tutor is mandatory.
- **Host Institution (HI) and External Research Institution (ERI)**
 - The **Host Institution (HI)** is the research center where the applicant carries out the research activity described in the application.
 - It must be an Italian research institute;
 - it can be profit or no-profit, private or public. Pharmaceutical and biotech companies are not eligible.
 - a declaration, undersigned by the HI head/responsible, must be provided by the applicant (see Additional documents).
 - The **External Research Institution (ERI)** is the center where the applicant carries out his/her training period.

- It can be an Italian or an international research center;
- it can be profit or no-profit, private or public. Pharmaceutical and biotech companies are not eligible.
- it must provide the spaces and equipment necessary to carry out the training period;
- if applicable, the applicant can provide a letter of intent of the head/responsible of the ERI where the training period will be carried out;
- a declaration, undersigned by the ERI head/responsible, must be provided by the applicant by the beginning of the project (see *Additional documents*).

5. Research team

- Only monocentric projects are admitted; no partners are admitted for this call.
- If required, collaborators are admitted. Collaborators are researchers with limited roles and functions in the project, they can be:
 - **internal collaborators**, part of the PI's research group;
 - **external collaborators**, researchers of external research institutions (Italian or foreign).
- External collaborators can request a cost recovery for a specific task (technical service, research or clinical consultancy – see *Budget section*).

6. Project duration and training period

- Only projects lasting 36 months are admitted for this call;
- a training period is encouraged at an ERI (Italian or foreign), and the related costs are allowed (see *Budget section*);
- the maximum duration of the training period is 6 months;
- the applicant must provide a declaration from the host institution where the research project will be conducted (see “Acceptance of the HI” in *Additional documents – Upload area* (Form 13);
- if already available, the applicant can provide a letter of intent in which the head/responsible of the ERI certifies his/her willingness to host the applicant for his/her training period. Please upload this document in “*Additional documents – Upload area* (Form 13);
- the “Acceptance of the ERI” is the agreement with the external research center. This document must be signed by the head/responsible of the ERI and must be provided by the start of the project. The head/responsible of the institute must report his/her willingness to host the applicant for his/her training period and must provide him/her with the necessary spaces and equipment to conduct the training period.

7. Budget

The budget description (Form 10) must be accurate and each item must be motivated and detailed per each research unit and per each year of the funding period. An inadequate budget description will lead to the exclusion of the project.

As a general rule, the maximum budget request for the GMSG cannot exceed 60.000 euros per year for a total of 180.000 euros for the whole project. The part of the budget dedicated to the salary can reach up to 31.000 euros per year, the part of the budget for the research can reach up 87.000 euros for the three-years project.

For the GMRF the part of the budget dedicated to the research fellowship can reach up to 25.000 euros per year, for a total of 75.000 euros for the three-years project, the part of the budget for the research can reach up 25.000 euros for the three-years project.

If the researcher has a permanent position from his/her host institution during the starting grant or the research fellowship, the contribution of the FFC Ricerca will no longer cover the costs of the salary. In this case the researcher is requested to contact FFC Ricerca's administration. Any exceptions can be discussed on a case-by-case basis.

Eligible costs

- Salary for the PI. FFC Ricerca will cover the cost of the research contract of the PI for the duration of the project. In case the applicant already has a contract with his/her host institution, FFC Ricerca can integrate it up to a maximum of 31.000 euros per year for the duration of the project for the GMSG, or to a maximum of 25.000 euros per year for the GMRF. If needed, please use the box in the *Additional notes* section (Form 12) to describe contract details.
- Small research equipment or accessories and software (justified and related to the current project).
- Consumables and animals.
- Participation in trip and scientific meeting (international conferences on CF)
- Costs to cover the training period (travel, accommodation, health/travel insurance).
- Publication costs, with clear reference to the project funded by this call.
- Costs for patients participating in clinical trials such as insurance coverage and travel costs.
- Overheads (general expenses not foreseen in the previous items, but in any case compatible with the admitted expenses): they cannot exceed 5% of the total budget.
- Collaborators cost recovery, such as external and occasional professional or technical services, costs for clinical consultancy or patentability analysis. The applicant can ask to use part of the budget to finance research activities carried out by one or more collaborators;

Ineligible costs

- Salary for internal or external collaborators.
- Furniture and stationery.
- Software not specifically related to the project.
- Basic laboratory or clinical equipment.
- Equipment repair or technical assistance costs.
- Office materials.
- Laboratory or clinical equipment for the ERI in which the researcher intends to carry out her/his training period.

8. Guidelines for filling in the application

The information requested in the application must be correctly reported in the respective forms as below.

Important note: please avoid past/copy of formatted text (such those from PDF files and from the Internet) which could create technical problems in downloading the final PDF of the application. We suggest you copy the text in a .txt file and, then, copy it in the application forms.

- **Form 1 - General information.**
 - Application details:
 - Project title;
 - Application: Type of Applicant, Type of Application and Type of call (GMSG or GMRF);
 - Number of researchers involved (including internal and external collaborators). For each collaborator, please provide a brief biosketch;
 - Research area;
 - Keywords that describe the project (as a suggestion, choose a maximum of 5 keywords from the list in Appendix 3)
 - Animals or human subjects involved in the project.
 - Applicant information and contract details; internal and external collaborators possibly involved in the project, the details of their host institution and their specific roles in the project. The acceptance of collaboration must be corroborated by personal declaration (see also point 7, *Additional documents*).
 - Enter here whether the training period is foreseen. If already available, the letter of intent can be uploaded in form 13 - *Administrative documentation - Upload Area*.
- **Form 2 - Project overview.** It must include: background/rationale, hypothesis and objectives, preliminary results relevant to the project, experimental plan and methods description, timing, anticipated output, relevance for FFC Ricerca mission.
- **Form 3 - Research Plan: Background, Specific Aims & Rationale.** The originality of the project must be clear from these items. The bibliography cited must be reported in this section.
- **Form 4- Preliminary Results.** They must be proved and convincing and refer to the results obtained by the applicant in preliminary investigations that bring the motivation and the justification of the proposal. The preliminary results will serve to demonstrate that the candidate has the capacity to carry out the proposed project. This part will be considered as absolutely necessary and decisive for the evaluation of the project. Images and graphs relating to preliminary results must be uploaded in this section as a single PDF document (max 25 Mb).
- **Form 5 - Experimental Plan and Methods.** In this part the following must be specified in detail: the experimental plan or clinical protocol, the methodology and materials intended to use, the justified number of the samples (whether patients or animals) to be examined and the statistical methods that will be required for the evaluation of the results. A description of the development phases of the project is also requested, including temporal ones (also with a Gantt chart), quality controls, and all pertinent references. In addition, the organization and management must be described to ensure the quality and feasibility of the project. Please detail the possible critical points and report any contingency plan (plan B). If applicable, describe in this section a realistic way to valorize the project results in terms of translational research. The Gantt chart and, if available, other images must be uploaded here as part of a single PDF document (max 25 Mb). Indicate in this form if it is a clinical project/trial.
- **Form 6 - Curriculum vitae.** Education and training, previous job and research experiences, significant publications in last 5 years (only in peer-reviewed journals only). For the GMSG, please report in this section at least 3 publications as first/last/corresponding author in original papers published in international peer-reviewed journals. For the GMRF, please report in this section at least 1 publication in

the field of cystic fibrosis in original papers published in international peer-reviewed journals.

- **Form 7 - Role and Contribution of the Applicant in the project.** Please describe the applicant's contribution to the project and how to coordinate the activities of the collaborators.
- **Form 8 - Features and facilities.** Applicant position title (if applicable); Main research fields; name of laboratory or clinical department and his/her responsible/chief and number of staff members; description of facilities (spaces, clinical resources, IT equipment, major equipment, facilities and services available). The technologies and services available for the implementation of the project must be detailed, indicating their specific relevance to each phase of the experimental plan.
- **Form 9 - Outside Collaborations/Services.** Describe the collaborators contribution to the project. External technical services or consultancy/professional services are to be considered. If required, facilities of FFC Ricerca must be reported in this section. External collaborators must provide a personal letter of commitment written and signed on the institutional letter-headed paper to be uploaded in the form 13 - *Administrative documentation – Upload Area*.
- **Form 10 - Budget.** Specify overall expenses and for each year (see *Budget* section). Any other financial supports must be indicated in this section, reporting the related details (description, amount, project title, granting agency). Note that the choice made in session “*General Information/Type of call*” corresponds to a specific budget table.
- **Form 11 - Lay Summary.** This must be written both in English and Italian (including Italian title), in a popular and well understood style. This summary is meant to serve as a succinct and accurate description of the proposed work; if the application is funded, this summary will be published in the “*Notiziario FFC Ricerca*” and in other media. The summary must state clearly the relevance of the proposed study to the FFC Ricerca mission (to promote innovative treatment and care for CF). In the Appendix 2 you can find some tips to help you write the lay summary.
- **Form 12 - Cover and Tutor/Mentor letters and additional notes.** Upload in this section the Cover letter and the tutorship or mentorship letters. Tutorship and mentorship letter must be written on the institute headed paper. If needed, use the “*Additional notes*” box to report any contract details.
- **Form 13 - Additional documentation – Upload area.** Documents to be uploaded: Acceptance of Host Institution, Acceptance of collaboration/Consent to Personal Data Processing, Acceptance of External Research Institution (ERI) for the training period (if applicable), Letter of commitment/Consent to Personal Data Processing - External collaborators and the required documents if a clinical/epidemiological study is proposed. In addition, use the dedicated optional box “*Referees*” in this form to provide any name of reviewers to suggest or to exclude, please indicate name, surname, affiliation and email.
- **Form 14 - Validate and Download PDF.** This section will highlight in red any empty fields or errors that can occur in filling the forms. By clicking on the red fields, the system opens the section that needs to be filled out. Download the application in PDF format by clicking on the “*Download/print this project*” button (at the left of the page). You can validate and download the application in PDF format at any stage. Once the application is completed (no errors are highlighted), click on “*Validate and send*” to complete the submission and send the application to FFC Ricerca's Scientific Direction.

8.1. Additional documents (See Forms 12 and 13 in the application platform)

8.1.1. Cover Letter

The application must be accompanied by a cover letter (see Form 12) summarizing:

- the general plan of the project;
- the reasons why it falls within the priorities indicated in the call for applications, in line with the objectives of the Foundation's mission;
- why it is believed that FFC Ricerca should consider the project worthy of funding.

If applicable, the applicants can also report in the cover letter any **patent registration**, completed or in progress if related to the proposed project, specifying available the details.

In case of **resubmitted projects** (previously submitted to FFC Ricerca and not approved), the cover letter must also include detailed, point by point, reply to the critiques of the referees if the previous application underwent full review. Resubmitted projects require extensive explanation, and cannot be resubmitted with only minor tweaks. A project can be resubmitted only once even if the proponent is different.

8.1.2. Host Institution (HI) and External Research Institution (ERI) documents.

- “Acceptance of the HI”: an agreement with the host institution, signed by the head/responsible of the HI must be provided; the head/responsible of the HI must indicate his/her willingness to host the applicant and must provide him/her with the necessary spaces and equipment to necessary to carrying out the research activity;
- Consent for use of personal data, according to the Italian Law 196/2003.
- Declaration of commitment signed by each internal and/or external collaborator involved in the project listed in the application.
- PI declaration of adherence to provisions governing laboratory animal care, if applicable.
- If applicable, the applicant can provide a letter of intent of the head/responsible of the ERI where the training period will be carried out;

8.1.3. Letter of commitment/Consent to Personal Data Processing - External collaborators

The letter must include the contribution of collaborator to the project. Each External collaborator will have to upload its own Letter of Commitment written on the headed paper of department or institution.

8.1.4. Clinical project

Projects that fall into this area of intervention must be clearly patient oriented. They must include, even just in part, diagnostic, therapeutic or rehabilitative interventions on humans, not provided for in common standard or from the personal plan of diagnosis, care and rehabilitation.

8.2. Documentation

According to the type of study, a clinical project must **provide documentation** in accordance with the current law:

- Clinical Trial^{**}: Regulation (EU) n. 536/2014
- Medical Device^{*}: Regulation (EU) n. 745/2017 and Regulation 746/2017
- Observational Study^{*}: Ministerial Decree of 30.11.2021 and (Italian Official Gazette G.U. No. 76 of 31 March 2008

^{**} The evaluation, authorization, and supervision of clinical trials are the responsibilities of EU Member States and European Economic Area (EEA) countries via the Clinical Trials Information System.

^{*}Ethical Committee Approval (for each partner/centre, if multicentre study) to be sent to FFC Ricerca after award of the grant and not later than 31st October 2024;

- Informed consent form (for interventions and for use of personal data, in anonymous form, for research purpose, released by patients or people involved in the study) plus patient information leaflet;
- Good Clinical Practice declaration by the applicant.

8.2.1. Methods and data management

For the preparation of a clinical application, FFC Ricerca strongly suggests to follow the procedures provided by the main international checklists, such as:

- CONSORT (for randomized studies)
- STROBE for observational studies)
- STARD (for diagnostic studies)
- PRISMA (for systematic reviews)

Further information are also available at the link of the [Equator Network Initiative](#).

Multicenter clinical studies are advised to use a web-based Case Report Form (CRF). Data management and monitoring could be more appropriately supplied by a Contract Research Organization (CRO). Given the relevant cost of CROs, FFC Ricerca could propose one which has been assessed as reliable and cost-efficient. Costs and agreement with the CRO have to be reported in the budget.

8.2.2. Projects including use of animal models

Any project which includes experiments on animals must be accompanied by a specific authorization of the Ethical/Technical Committee of the Institute hosting the animal facility to be submitted only after award of the grant and not later than 31st October 2024. Moreover, the PI or coordinator must declare that the procedures concerning those experiments will follow the instructions included in the legislative decree 2014, n. 26, “Attuazione della direttiva 2010/63/UE sulla protezione degli animali utilizzati a fini scientifici (14G00036) (GU n. 61 del 14- 3-2014)”.

NOTE: *If the approval of Ethical Committee (for clinical trials) or Ethical / Technical Committee (for animals) and “Parere Unico” are not available by 31st October 2024, a follow-up report on the ongoing application either to the Italian Ministero della Salute or the Local Ethical Committee must be sent to this Foundation by email. Providing this information to the FFC Ricerca Foundation is crucial for the correct management of the grant.*

8.2.3. Resubmitted projects

Researchers who are going to resubmit a project previously not funded by FFC Ricerca, even with a different title and improvements, must follow these recommendations:

- The **cover letter** must highlight the relevant modifications of the revised project.
- If the previous application underwent full review, the cover letter must also include detailed, point by point, reply to the critiques of the referees and FFC Ricerca Scientific Board.
- In case the revised application is submitted by a different PI, an explanation must be provided in the cover letter.
- The same research project can be resubmitted only once, even if substantially modified and even by a different proponent.

8.2.4. Submission by former FFC Ricerca grant recipients

A former FFC Ricerca grant holder may submit a new project or a proposal of development of a project already funded by FFC Ricerca (“Renewal project”). In both cases all of the following must apply:

- The previous project has been completed and its final scientific report has been already submitted to FFC Ricerca. The scientific report must include details of the project’s achievements, the contribution of each Partner (for multicentre projects) and a list of the resulting publications and congress presentations (abstracts).
- Coordinators or partners in a multicentre project financed by FFC Ricerca may submit a new research proposal as PI or Coordinator provided their previous projects were completed or that the ongoing project expires on 31st August 2024, and that they are not involved in other FFC Ricerca projects.

8.2.5. Tutorship and Mentorship letter

The tutorship letter, for the GMRF must report modalities that the tutor will adopt to direct and train the young researcher during his/her research period. The mentorship letter, for the GMSG must report the mentor’s modalities to support the young researcher in his/her research period. Note that the choice made in session “General Information/Type of call” corresponds to a specific letter to be attached.

9. Evaluation of applications

Important note: incomplete or behind schedule applications will not be processed for evaluation.

Procedure of evaluation

Grants will be awarded on a priority basis. Specific factors that will play a major role in determining a successful outcome of the application are:

- relevance to the Italian CF Research Foundation’s mission and to the priority areas (see point 1);
- soundness and originality of the study;
- relevance of the preliminary results;
- the potential value to improving the clinical and care strategies;
- the potential value to stimulating further studies, mainly on translational basis;

- the appropriateness of the design of the study;
- the scientific record of the participants;
- methods reliability*;
- feasibility within the duration of the project;
- facilities appropriateness;
- clarity and quality of lay summary.

*For projects involving people with CF, healthy individuals or animals, their number and the consequent statistical analysis will be taken into strong consideration.

All accepted applications will undergo a preliminary review by the Scientific Committee of the Italian CF Research Foundation on the basis of their relevance to the Foundation's mission and overall quality. Projects selected in this triage step will undergo full peer-review by an international panel of experts. In the final step, the projects will undergo evaluation by the Scientific Committee, taking in due consideration the independent referees' comments. The Scientific Committee will also review the research activities related to the required budget and the project duration. **If the proposed activities and the budget are not considered consistent with the duration, the project will be rejected. The evaluation of the Committee is final, with the approval of the Board of Governors.**

Due to the competitive nature of project selection, projects that have received a positive evaluation by the external reviewers may be denied funding. Co-funding of projects must be declared and accurately described in the appropriate section of the application (see Form 10).

10. Fellowship and research grants

- FFC Ricerca reminds that the project must be carried out mainly with the direct involvement of PI and internal and/or external collaborators as indicated in the application.
- FFC Ricerca will directly manage the research grant. Exceptionally, other modes of managing the grant and research collaborators must be discussed with the FFC Ricerca. Upon awarding the grant, FFC Ricerca will provide the PI with detailed information on the procedure to follow.
- Principal Investigator must participate at the Annual Convention of the Italian CF Researchers as guests of FFC Ricerca. Participation in the entire Convention is mandatory because it is a working time on research funded by FFC Ricerca and not just a general updating conference.

11. Awarding and management of research funds

The allocation of funds will be formally decided by the Board of Governors of the FFC Ricerca, and communicated at the time of award. As a rule, funds are disbursed to the PI and not to the Institution where he/she intends to carry out the funded project.

FFC Ricerca will manage directly the funds according to the PI's indications. Approved PI must maintain an accurate and up-to-date administrative account, parallel to FFC Ricerca. With reference to budget indications, expenses will be administered per year.

Award recipients will be required to provide a detailed annual administrative report by the end of the first and by the end of the second year to obtain subsequent payments.

Any changes of the original destination of budget formalized at the time of assignment, occurring during the completion of the project, must be exceptional and formally requested and agreed with FFC Ricerca.

FFC Ricerca will not pay any expenses made after the date of conclusion of the project or in excess of the assigned budget. It is not possible to issue expense orders in the last month of the project. Any costs exceeding the budget will be charged personally to the PI.

12. Scientific and administrative reports, publications

At the end of each year of activity the PI must provide a detailed scientific and administrative progress report, which is necessary to decide the continuation of the funding. At the end of the project, the investigators are invited to submit, together with the administrative report, a final scientific report including any publications and congress presentation abstract referring to the project. Any publication or congress abstract relevant to the project must be forwarded to the Foundation before such reports to be published.

No publication or dissemination of the results of ongoing research should jeopardize the future patenting of any research results as a form of pre-dissemination.

The Fondazione per la Ricerca sulla Fibrosi Cistica/FFC Ricerca must be acknowledged in all publications deriving from the funded project (congress abstracts, book chapters, scientific articles, congress slides, press releases, etc.) specifying the code of the relative grant and by inserting the FFC Ricerca logo both on the slides and the posters of the congress. Furthermore, the adopters of a project, as indicated by FFC Ricerca, have to be mentioned (see Progetti di ricerca on FFC Ricerca website).

FFC Ricerca may ask investigators to collaborate to public commitment and dissemination of the results of their research in order to support the fundraising of FFC Ricerca. To this end, it is up to FFC Ricerca to contact the investigators.

13. Research results, intellectual property and patents

One of the main goals of FFC Ricerca is to translate research findings into clinical applications available to CF patients. Sometimes, this can be achieved by partnering with the industry, so that the most promising research results can be fully developed into therapies, devices and diagnostics.

FFC Ricerca requires that all scientific results derived from projects financed by FFC Ricerca, which are of importance for a possible development, are evaluated for the purposes of intellectual protection and/or commercial valorization.

Funded scientists must promptly notify FFC Ricerca in writing of the intention to file any patent and the execution of agreements with for-profit entities relating to the results of research funded by FFC Ricerca.

The patent application relating to results from projects funded by FFC Ricerca must first be discussed and authorized by FFC Ricerca. The dedicated institutional offices of the founded investigators (TTOs - Technology Transfer Offices) can provide support and assistance on intellectual property matters and technology transfer activities.

In any case, the intention to file a patent application must be previously communicated to FFC Ricerca, in time to allow negotiations between TTOs of the PI's/Collaborators Institutions and FFC Ricerca regarding evaluation of the expenses incurred by each of them and the percentage of ownership of the patent of each of the parties, both in term of expenses to sustain it and in terms of possible future revenues. The relevant agreement with the funded scientists' institutions shall be negotiated by the parties in good faith.

FFC Ricerca reserves the right to participate in the ownership of any know how, intellectual property and inventions derived from funded projects, proportionally to its investments. The funded researchers must promptly inform FFC Ricerca in writing of the intention to file any patent and of any proposal by for-profit entities related to FFC Ricerca funded research results.

FFC Ricerca is confident that funded researchers will operate with clarity and honesty regarding the attribution of relative merit to any work, invention or discovery. Researchers must always remember that all funds supporting CF research are raised through voluntary donations.

14. Writing and submitting applications

Applications must be written in detail and submitted only through the dedicated online platform <https://forms.fibrosicisticaricerca.it/en/>, other options are not accepted. For first time access a registration is required to create a personal account and allow applications submission. Attention must be paid to not exceed the indicated number of characters in each form. Any images (photos, graphs, tables) must be low-resolution version and uploaded in one single PDF that must be attached in each specific section of the platform if required. Applications must be submitted through this platform by midnight of **31st January 2024**.

15. Appendixes

15.1. Appendix 1 - The FFC Ricerca facilities

15.1.1. SCP – Primary Cell Facility (Servizio Colture Primarie)

Established in 2012 the primary cell facility, born from the collaboration between the FFC Ricerca and the Medical Genetics laboratory of the Giannina Gaslini Institute in Genoa, provides a collection of **cell cultures obtained from bronchial epithelium of both CF patients and non-CF** control subjects to CF researchers. The bronchi, from which the cells are isolated, come from the transplant center in Milan (Thoracic Surgery Unit, Polyclinic of Milan).

The **aims** of the facility are:

- the study of the pathophysiology of cystic fibrosis.
- the evaluation of therapeutic strategies.

The **cells and available services** are:

- collection of primary bronchial cultures isolated from bronchi of explanted lungs from individuals undergoing lung transplantation (CF patients or subjects transplanted for other pathologies)
- protocol for the correct cultivation of the submitted cells;
- training in SCP's laboratories.
- advanced tests depending on researchers requests and the expertise acquired by the lab.

The following is a list of cells **genotypes** available in the facility:

| | | |
|-------------------------|------------------------|----------------------|
| F508del/F508del | F508del/3878delG | N1088D/G542X |
| F508del/G542X | F508del/1874insT+Y577F | N1303K/2183AA>G |
| F508del/R1162X | F508del/L927P | N1303K/711+5G>A |
| F508del/1717-1G>A | F508del/C276X | R1006C/M1V |
| F508del/N1303K | F508del/L1077P | R1158X/3849 +10KbC>T |
| F508del/R553X | F508del/2789+5G>A | R1162X/2789+5G>A |
| F508del/CFTRdelE 17A-18 | F508del/Q552X | R1162X/3849+10KbC>T |
| F508del/3849+10KbC>T | G542X/711+5G>A | del Ex 22-23-24/UK |
| F508del/62+1G>T | G542X/H609R | 1525-1G>A/G458R |
| F508del/G85E | G542X/1717-1G>A | 2789+5G>A/M1V |
| F508del/2184insA | I502T/N1303K | 2789+5G>A/R1070Q |
| F508del/1259insA | | |

For further information, please write to: serviziocoltureprimarie@fibrosicisticaricerca.it

15.1.2. CFaCore - Cystic Fibrosis animal Core Facility

Established in 2009, CFaCore is a specialized research facility providing services to CF researchers. Located at San Raffaele Hospital in Milan, it operates within a dedicated infrastructure with a Biosafety Level 2 (BSL2) animal facility. The core facility manages CF murine colonies and conducts pre-clinical research to advance new treatments for CF.

Key services are offered:

- management and distribution of transgenic cystic fibrosis mice, ensuring their well-being for experimental purposes;
- scientific and regulatory support to plan pre-clinical studies and prepare documentation for ethical approval;
- pre-clinical models of acute and chronic respiratory infections and inflammation established with reference and clinical CF-related pathogens along with associated virulence factors such as LPS;
- execution of experimental protocols including the administration of systemic or aerosol pharmacological treatments according to specific short or long-term schedules;
- analysis of pathogen and host responses, incorporating lung function measurements to generate comprehensive results;
- definition of milestones and project goals to drive efficient progress and ensure the successful achievement of overall objectives.

For further information, please contact CFaCore coordinator Dr.ssa Alessandra Bragonzi, Infections and Cystic Fibrosis Unit, San Raffaele Scientific Institute, bragonzi.alessandra@hsr.it.

15.1.3. CFDB - Cystic Fibrosis DataBase - www.cfdb.eu

Established in 2011, the CFDB is a web-based, free access tool for health care professionals, researchers and students to evaluate in real time what are the current evidences about clinical efficacy of interventions in CF.

The CFDB collects more than **1,300 studies divided in 8 sections**, including Cochrane reviews, Cochrane protocols, DARE, HTA and Economic reviews, published RCT, published non-RCT, congress abstracts and ongoing trials. In addition, CFDB collects **50 thematic**

worksheets, named *Topics*, on relevant clinical subjects in CF that critically summarize the state of the art of available evidences.

The objective of CFDB is to classify clinical studies to get answers to specific questions:

- which interventions are effective, in which groups of CF patients and for which outcomes?
- to what extent do the results of the literature allow to make decisions for specific clinical issues? What issues need to be studied further?

This tool may help clinicians, researchers, students to have a faster updated view of clinical research in CF by using queries on the main topics in CF care. It could also be helpful to anyone going to design new studies, as it provides a concise description of what is currently known and what issues, on the contrast, need additional research.

What can you do with CFDB?

- You can build a query, selecting terms from search menus;
- You can also select one or more citations and read the details of the studies;
- You can read updated summaries (Related topics) on the state of the art of the most relevant topics in CF.

For further information, please contact CFDB coordinator Roberto Buzzetti at robuzze@gmail.com.

15.2. Appendix 2 - How to write a lay summary

What is a lay summary?

A lay summary is a **brief paragraph about your research project**. It explains complex ideas and technical terms to people who do not know about the subject, or a **lay audience**. The audience of FFC Ricerca includes everyone from non-specialists in your field to volunteers, patients, caregivers and the general public.

The lay summary scheme

The lay summary should be no longer than 3500 characters with spaces included, and should consist all of the following sections and questions to answer:

- *Title (only in the dedicated space of the form)*
- *Lay Title [pleas avoid acronyms, e.g. cystic fibrosis not CF] (only in the dedicated space of the form)*
- *What is your scientific question?* [max 300 characters with spaces included]
- *Why is this important?* [max 700 characters with spaces included]
(Explain the impact of the work, what is going to change)
- *How will you conduct the research?* [max 700 characters with spaces included]
(Methodological information of how you will carry out your research project)
- *What do you hope to achieve?* [max 700 characters with spaces included]
(Summary of the most important anticipated results)
- *What are the implications of your research for persons with cystic fibrosis? Are there any reasons for caution?* [max 700 characters with spaces included]
- *Future perspectives* [max 400 characters with spaces included]

Instructions to authors:

- Make the summary easy to read and interesting. Don't oversimplify. While trying to make it simple, ensure that the crux of your research is not missed out. The reader must clearly understand what the research is about and how it will affect the society.
- Attempt to keep the 'reading age' of your summary at high school level.
- Use first person and active voice ("we agreed" rather than "it was agreed").
- Use positives not negative sentences.
- Keep sentences short, clear and focused.
- Do not overstate the importance/relevance of your study. Place it honestly within the existing literature and how the study adds to current knowledge.
- Avoid jargon and scientific abbreviations (e.g. FEV₁) unless absolutely necessary. Technical terms and complex mechanisms/measurements need to be thoroughly explained using basic terminology. Acronyms should be used sparingly and must be spelled out on first reference.

15.3. Appendix 3 – List of keywords

In the appropriate section of Form 1 - General information, briefly describe the project using the following keywords list as a suggestion. Choose a maximum of 5 keywords.

| | |
|--------------------------------------|--|
| 1. Modulator Therapies | 22. Diabetes |
| 2. Gene therapy | 23. Orphan mutations |
| 3. <i>Pseudomonas aeruginosa</i> | 24. Neonatal screening |
| 4. Non-tubercular mycobacteria (NTM) | 25. <i>Aspergillus fumigatus</i> |
| 5. Psychological aspects | 26. Metabolomics |
| 6. Epidemiology | 27. Biomarkers |
| 7. Anti-inflammatories | 28. Genomics |
| 8. Mouse models | 29. CF alternative targets |
| 9. Cell models | 30. Microbiome |
| 10. Therotyping | 31. CFTR and basic defect |
| 11. Other experimental models | 32. Autophagy |
| 12. Proteomics | 33. Multiresistance |
| 13. Interactomics | 34. Clinical studies and translational medicine |
| 14. Lipidomics | 35. RSV - Respiratory syncytial virus |
| 15. Bioinformatics | 36. Electrophysiology |
| 16. Mitochondria | 37. Chronic obstructive pulmonary disease (COPD) |
| 17. Phagic therapy | 38. Lung transplantation |
| 18. Gene modifiers | 39. Pharmacology |
| 19. Mucolytics | 40. Lung Disease |
| 20. <i>Burkholderia cenocepacia</i> | 41. Bone disease |
| 21. Imaging | 42. Liver disease |