

Full Application

Summary

Reference number	
Institution	
Lead Applicant	
Total Requested	€

General Information

Project Title

Retrieved from the Letter of Intent.

Project duration

(min 12- max 36 months) - Retrieved from the Letter of Intent.

Funding Opportunity

Retrieved from the Letter of Intent: FT General Grant, FT Career Award, Fondazione Pisana per la Scienza/Fondazione Telethon (FPS/FT)

Type of Applicant

Retrieved from the Letter of Intent:

- New Applicant is a researcher who has never applied to a Telethon Call; he/she may only submit a New Application.
- Former Applicant is a researcher who has already applied to a Telethon Call but has never been funded; he/she may submit a New or a Revised Application submitted during the previous 2019 call (GGP19).
- Former Grantee is a researcher who has already been funded by Telethon in the past; he/she may submit a New, a Revised or a Renewal Application.

Please note that in case of a wrong classification within the Letter of Intent, Telethon has modified and corrected ex officio the Type of Applicant/Application.

Type of Application 1

New Applicant - may only submit a New Application.

Type of Application 2

Former Applicant - may submit a New or a Revised Application

Type of Application 3

New Application

Former Grantee - may submit a New, a Revised or a Renewal Application

Previous Application Number (Former Applicant)

Type the previous Application Number and select your previous role in case of:

- Revised Application
- Renewal Application

Retrieved from the Letter of Intent

Applicants submitting a Revised Application must fill in the Cover Letter form in the dedicated section

Reference: Telethon_

(see page 5).

Previous role 1

Previous Application Number (Former Grantee)

Type the previous Application Number and select your previous role in case of:

- Revised Application
- Renewal Application

Retrieved from the Letter of Intent

Applicants submitting a Revised Application must fill in the Cover Letter form in the dedicated section (see page 5).

Previous role 2	
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5011

Forename(s) Surname

Institution County

City/Town

Position
Department
Email Address

Overview

А	bs	tra	Ct

(max 250 words) - Provide the following information:

- Broad objectives and specific aims
- Background/Rationale
- Research design and methods for achieving the stated objectives
- Anticipated output

(max 250 words)

Summarize the project using an English lay language. This description is very important, it is meant to describe the project to lay people during the review process and will eventually serve for lay communication reasons after award and, as such, it may become public information. Therefore, do not include proprietary/confidential information.

Lay Abstract in italian

(max 250 words)

Summarize the project using an Italian lay language. This description is very important, it is meant to describe the project to lay people during the review process and will eventually serve for lay communication reasons after award and, as such, it may become public information. Therefore, do not include proprietary/confidential information.

Please consider that the lay abstracts are fundamental parts of the application and Fondazione Telethon may slightly modify both Lay Abstract texts for communication purposes and that these information will be available on Fondazione Telethon website in case of projects approved for funding.

Retrieved from the Letter of Intent, if present.

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MeSH Terms	
Disease Name	
Retrieved from the Letter of Intent, if pre	esent.

ORPHA Number

Retrieved from the Letter of Intent, if present.

Orphanet classification	
Retrieved from the Letter of Intent, if pre	esent.
Disease OMIM Number	
Retrieved from the Letter of Intent, if pre	esent.
ICD-10 Code	
Retrieved from the Letter of Intent, if pre	esent.
No Area of Research have been added	
Retrieved from the Letter of Intent, if pre	esent.
No Research Type have been added	
Retrieved from the Letter of Intent, if pre	esent.
No Research Step have been added	
Retrieved from the Letter of Intent, if pre	esent.

Cover Letter

Attach the Telethon Review Report of the previous Application in this section. If needed, contact the Telethon scientific staff (telethonscience@telethon.it).

Cover Letter

(max 15,000 characters) - If the previous Application was excluded by Triage, the Cover Letter must highlight the relevant modifications made. If the previous Application underwent Full Review, the Cover Letter must include a detailed reply to the critiques.

If the Applicant is different from the previous Application, the reason must be provided in the Cover Letter.



Scientific Approach

Hypothesis, Background and Rationale

(max 5,000 characters) - State the main hypothesis to be tested and explain the impact of the problem addressed by the proposed project. Critically evaluate the existing knowledge and identify the specific gaps to be filled to progress in the relevant field.

Overall Objectives

(max 1,000 characters) - Describe the overall objectives that the proposed research is intended to accomplish.

Preliminary Results

Provide an account of preliminary unpublished studies performed in the Applicant's laboratory relevant to the proposed research. Preliminary data are an essential part of a research grant Application, as they aid the assessment of the likelihood of success of the proposed project.

Results are considered 'preliminary' only if unpublished.

(max 10,000 characters) - Provide an account of preliminary unpublished studies performed in the Applicant's laboratory relevant to the proposed research. Preliminary data are an essential part of a research project Application, as they aid the assessment of the likelihood of success of the proposed project.

Results are considered 'preliminary' only if unpublished.

List the specific Aims (click on **Add** button for each Aim) of your project.

For each aim fill in the required information (fields):

- Title
- **Brief Description** (max 1,000 characters per aim) What is the question being asked? What is the general experimental design?
- **Experimental Plan** (max 3,000 characters per aim) How are you going to address this aim? Please provide an extensive description of the experimental approaches.

If the study involves vertebrate animals, please refer to the "**Telethon rules and policy on animal experimentation**" section on the following page.

Explain the need for **collaborations** (if any) to achieve the scientific aims of the proposed project. Indicate how the idea of collaborating originated, the different approaches each collaborator will bring to the overall study, and how the collaboration will be conducted. Include an explicit description of the collaborative elements that are essential for the project to be carried out. Collaborators are expected to have research experience and must have an established record for independent research.

Any collaboration must be listed in the specific section (see page 9).

Please note that Telethon also funds a **Network of Genetic Biobanks (TNGB)** whose purpose is to collect, preserve and offer to the scientific community biological samples and related clinical data from individuals affected by genetic diseases for research purposes. Refer to the online catalogue of the TNGB (http://biobanknetwork.telethon.it/), to identify potentially useful biological samples.

Refer to the "Figures" section (page 3 of this document) to create and upload the Figures' PDF file.

Significance and Innovation

(max 2,000 characters) - Describe which important problem will be addressed in the proposed study and how the scientific knowledge will be advanced, if the aims of the project are achieved. The objectives of the study must represent a significant step forward beyond the current state of the art and include substantial original work. Indicate if the project employs novel concepts, approaches or methods and if it challenges existing paradigms in the field or develops new methodologies or technologies.

Impact on patients and relevance to Telethon's mission

(max 2,000 characters) - Describe how close to therapeutic development, or to any other potential impact on patients, the proposed studies are. Clearly specify how the goals of the project fit with Fondazione Telethon's mission (http://www.telethon.it/en/what-we-do/our-mission).

Refer to the Figures section (page 3 of this document) to create and upload the figures' PDF file(s).

HUMAN SUBJECT

VERTEBRATE ANIMALS

Indicate whether the Study involves:

If the grant is approved for funding, funds WILL NOT BE PROVIDED until the pertinent Ethical Documentation has been obtained.

Please activate in due time all necessary procedures to obtain this approval in accordance with the relevant Italian laws (https://www.aifa.gov.it/modulistica-sperimentazione-clinica)

Telethon reserves the right to ask for a copy of all the relevant approval documentation.

Indicate whether the study involves:

- 1. Human samples from a collaborator site or an external
- 2. Human samples from individuals referred to the PI's Host Institution
- 3. Individuals enrolled in clinical trials
- 4. No human samples or subjects.

Should the grant be approved for funding, funds will not be provided until the pertinent Ethical documentation has been obtained. Please activate in due time all necessary procedures to obtain this approval in accordance with the relevant Italian laws (https://www.aifa.gov.it/modulistica-sperimentazione-clinica). (https://www.aifa.gov.it/modulistica-sperimentazione-clinica).

Be aware that the relevant approval documents must be provided for grant activation.

Does your proposal involve vertebrate animals?

Specify whether or not activities involving vertebrate animals are planned at any time during the proposed project.

Telethon rules and policy on animal experimentation

Telethon recognizes that experiments on animals are often necessary in many areas of biomedical research. Proposals submitted for the evaluation MUST explain why the scientific objectives cannot be achieved without using animals.

Where experiments using animals are necessary, you are required to strictly adhere to the relevant Italian laws, rules and regulations (D.to L.vo 26/2014); moreover, **approval by your Institution Ethics Review Body is mandatory**. The ethical review process is a means of ensuring that any use of animals within lab animal facilities is carefully considered, adequately justified and carried out as humanely as possible, so that any adverse effects experienced by the animals are more than offset by the benefits that arise from the study.

Measures should be put in place to avoid unnecessary duplication of research/testing and fully implement the **Three Rs** (Reduction, Replacement and Refinement, from The Principles of Humane Experimental Technique, Russell and Burch, 1959), from the moment it is recognized that an animal experiment will take place, through the period where the animals are sourced and arrive at the facility, and up to the time they are either dead or have been re-homed. This includes optimizing standards of animal husbandry and care and effective training, supervision and management of all personnel involved. Microbiological status is important not only because there are welfare imperatives in minimizing the incidence of disease but also to avoid the risk that subclinical infections affect research results.

Provide a detailed description of the proposed use of the animals in the work outlined and identify the species, strains, ages, and sex of animals to be used in the proposed work. Provide information on the veterinary care of the animals involved.

Make sure that the fewest animals compatible with obtaining a valid scientific result are used. In this regard, in planning your experiments you should carefully estimate the number of animals needed. You should take into account the likely magnitude of the effect you will be studying and the frequency with which that effect will be achieved for given levels of statistical significance and power. It is unacceptable to base the number of animals to be used solely on the calculation of the number of experiments that can be carried out at any given time. It is also unacceptable to state that the numbers are based on "previous experience" without additional justification, or to answer the question on numbers of animals to be used by paraphrases such as "these numbers are chosen as the minimum necessary to achieve statistical significance". Too few animals is just as unsatisfactory as too many.

Be aware that the relevant approval docs must be provided for grant activation.

(Cited Literature



(max 20,000 characters)

List all references. The list must include the name of all authors, year of publication, title, book or journal, volume number and page numbers. If a bibliographic management software is being used, the format of the journal "Developmental Dynamics" may be applied.

Concise references are not allowed.

The complete list of references will be visible to Reviewers at any evaluation phase.



Previous Achievements

Project number and title of the most recent Telethon grant	

Previous achievements of the most recent Telethon grant



Feasibility, Timing, Clinical Protocol

Please upload a GANNT chart (in PDF format) describing the timeframe foreseen for the different Specific Aims and their components.

If applicable, clearly define:

- 1. Study design, i.e. blind, double blind, open, etc.
- 2. Study population, i.e. planned number of patients, inclusion and exclusion criteria, etc.
- 3. Description of the clinical procedures/medical examinations planned and the time interval between them State the potential difficulties and limitations of the proposed procedures and discuss alternative approaches to overcome them.
- 4. Study medication(s)/drug(s) (if applicable): dosage, administration, blinding, etc.
- 5. Safety: define adverse events and how they will be monitored; describe potential risks (physical, psychological, social, legal, or other) and assess their likelihood and seriousness; indicate if psychological support to patients is available. Describe alternative treatments and procedures (where appropriate) that might be advantageous to the subjects. Provide information about the Data Safety Monitoring Board that will be set in place.
- 6. Data management and statistical plan; discuss how data will be collected, analysed and interpreted. Describe in detail the statistical methods to be employed.
- 7. Provide the timetable of the study.

A clinical project must be supported by an Ethics Committee's approval in accordance with the laws of the Italian Ministero della Salute (http://www.aifa.gov.it/content/sperimentazione-e-ricerca).

NOTE: If the clinical protocol is already available, it has to be **uploaded** in this section. Otherwise, if the study is funded, the protocol and related documents must be provided to Telethon in order for funds to be released.

In case of doubts, please contact the Telethon scientific staff (<u>telethonscience@telethon.it</u>) before submitting the final Application.

NGS

Next Generation Sequencing

Do you plan to perform Next Generation Sequencing (NGS) experiments?

If the Applicant intends to perform NGS experiments, he/she is asked to provide additional information and to fill in the pertinent fields.

Organism Name

(max 250 characters) - provide the name of the organism target of sequencing. For example: Homo sapiens, Mus musculus, Drosophila melanogaster, etc.

Estimated number of samples and/or runs

provide an estimated number of samples to be sequenced or the number of sequencing runs foreseen in the project.

Type of Experiment

please describe the type of sequencing approach. Choose the appropriate option from the following list:

- Whole Genome Sequencing (WGS)
- Whole Exome Sequencing (WES)
- Transcriptome analysis
- Epigenomics
- Metagenomics

Or, if not present in the list, provide a brief description (e.g. amplicon or custom target sequencing, etc.) in the field dedicated to **other types of experiments** (max 250 characters).

Other Types of Experiments

(max 250 characters).

NGS Platform

provide the name of the NGS platform to be used. Choose the appropriate option from the list:

- Illumina (MiSeg, HiSeg, Genome Analyzer, etc.)
- Ion Torrent (PGM, Proton)
- Third Generation/Single Molecule Sequencing

Or, if not present in the list, provide a brief description in **other NGS platforms** (max 250 characters).

Other NGS Platforms (max 250 characters).

Personal Data

Click on "Add Publication" to select **up to further 10 peer-reviewed publications from the list of publications already recorded in the Applicant's account. All references** relevant to the present Application **need to be** marked with an asterisk (*) in the Publications section of the Applicant's account.

ID Research Platform

Indicate one of the Researcher Platforms and provide your personal author ID. If you do not have one, we suggest you to generate an ORCID ID (http://orcid.org/).

Personal Author ID

Indicate one of the Researcher Platforms and provide your personal author ID. If you do not have one, we suggest you to generate an ORCID ID (http://orcid.org/).

Financial Interests Disclosure

(max 1,000 characters) – Declare all possible financial conflicts of interest that might be perceived as relevant. Financial interests will not invalidate the Application, nor will they automatically disqualify it from being evaluated.

Title
Forename(s)
Surname
Date of Birth

Surname
Date of Birth
Nationality
Institution

Address Line 1
Address Line 2
Address Line 3

County
Postcode
Department

Employment, Research Experience, Scientific Career and Publications are automatically embedded from the Applicant's account.

Education

From	Degree	Class	Country	Subject	University/Institute

Employment

Start Date	End Date	Job Title	Employer	

Grants

Start (mm/yyyy)	Duration	Currency	Total Award Amount	Source	Reference	Туре	Title	Role of Applicant	Details

Experience

Experience	Notes	Date

Scientific Career

Scientific Career		



Collaborations

ACTIVE COLLABORATORS	<u> </u>			
Name				
Title			Address Line 1	
Forename(s)			Address Line 2	
Surname			Address Line 3	
Date of Birth			County	
Nationality			Postcode	
Institution				
Department				
Laboratory	гу		A 0	
Contribution to the Project				
PDF Scan of the Collabor (in English only). Please note any filenames saved without diacritical lett **Please be advised that t digital signatures with an	with diacritical ma ters / accents. the system does	not support	digital signature uploads.	
OTHER COLLABORATORS				
First Name	<u> </u>			
Last Name				
Institution				
Department				
Laboratory				

Budget and Host Institution

Total Budget		
	Total	
Total		

Organisation Approver

The Organisation Approver will have to be a person (Institution's Director or Responsible Official or Administrative Representative) who, representing the Organisation where the research project will be performed, will have the responsibility for the Application Approval and final Submission.

For the FPS funding opportunity please indicate Dr. Leopoldo Laricchia Robbio (llaricchia@fpscience.it) as Approver.

Click on Add Contact to invite the Organisation Approver, who will be notified by mail.

If the Organisation Approver is not already available in the menu in the IntelliSence menu, add the new one clicking on **Add Person** and save it. Personal email accounts are not accepted, please make sure that the Organisation Approver's email is the **institutional account**.

Please confirm that you wish to include this Organisational Approver then click on **Send the Invitation**. In the Organisation Approver box (in the Budget and Host Institution section) the Approver's details and the approval status are shown; in case of error the Approver can be deleted clicking on the **Remove** button.

The selected Organisation Approver will receive an email alerting that she/he has to take on the Application Approver role.

Title

Forename(s)

Surname

Honours Address

Institution
Department

Telephone No. Email Address

Reviewers

Suggested Reviewers

Please provide details of any Suggested Reviewers for your grant application.

The Applicant may suggest external referees - **not currently working in Italian Institutions** - expert in their own fields of research, who could competently review the Application. Co-authors in scientific publications and/or individuals who have been associated with the Applicant and/or his/her collaborators within the last 5 years should be avoided.

Telethon reserves the right to choose external referees independently.

Excluded Reviewers

Please provide details and reason of any Reviewers that should be excluded from your grant application.

Should the Applicant prefer to **exclude direct competitors** from being chosen as reviewers, their names can be indicated here. If the indications were not clearly justified, Telethon will disregard any exclusion request.



Notes

Your Notes, if any

Any personal comments, details or additional information the Applicant wishes to add to any specific sections of the Application can be inserted here. Please indicate which section you are referring to and the reasons for including more information.



Declaration

Full Name

The Applicant has to declare that the information included in the online Application is accurate and complete, and that he/she complies with Telethon's terms and conditions. The Applicant must also agree with the personal data treatment for Telethon's institutional purposes (Italian law 196/2003).

If the Declaration and Privacy Statement is not filled in by the Applicant, the Application will not be processed for review.

Place/Date

The Applicant has to declare that the information included in the online Application is accurate and complete, and that he/she complies with Telethon's terms and conditions. The Applicant must also agree with the personal data treatment for Telethon's institutional purposes (Italian law 196/2003).

If the Declaration and Privacy Statement is not filled in by the Applicant, the Application will not be processed for review.



Full Name	
Central Hypothesis, Background and Rat	ionale
Objective(s) The "why" of the project.	
Research Plan The "what" and "how" of the project.	
Importance and Novelty	
Preliminary data	
Brief biosketch (to be written without picking data from the CV is	nfo in the Contact section)
Personal Statement (why the applicant is well-suited for the role in the previous experimental work on the specific topic environment; and past performance in the field.)	ne proposed project. The relevant factors may include aspects of training; c or related topics; technical expertise; collaborators or scientific
Full Name (LOI)	
he/she complies with Telethon's terms and condi- Telethon's institutional purposes (Italian law 196/	included in the online Application is accurate and complete, and that itions. The Applicant must also agree with the personal data treatment for /2003). Iled in by the Applicant, the Application will not be processed for review.
Place/Date (LOI)	

If the Declaration and Privacy Statement is not filled in by the Applicant, the Application will not be processed for review.

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Telethon's institutional purposes (Italian law 196/2003).

Reference: Telethon_