

FONDAZIONE




## Telethon Rare Diseases and COVID-19 Special Grant

# GUIDELINES for preparing and submitting the Online APPLICATION

### INTRODUCTION

The Application form is available on **TETRA – The Telethon Project Management system portal** at <https://projects.telethon.it>.

**Registration.** To register, fill in and submit the Application, refer to the *TETRA Portal Instructions.pdf*  available on the Home page.

Please click on the **Register** button and enter your email address: to complete the registration process follow the online instructions. After the first registration, you can change your email address, if you wish to.

If you have questions concerning the Application, click the **Contact Us** link on the left hand menu to send a message.

**Personal Details.** Before proceeding to complete an Application form, please check the **Manage My Details** on the left hand menu of the Home page and update your **Basic Information and Update CV** sections. All this information will automatically populate the relevant fields of your Applications.

In the **Manage My details-Update CV** section ensure to update the following items: **Education/Training, Positions, Personal Statement/Scientific Career**, as all these are required for the submission of the Application. For the **Publication** section, please go to My Research Output section on the left hand menu and refer to the instruction in the *Tetra Portal Instruction.pdf* document.

**You will not be able to edit this information directly from the Application form;** but you can return to the *Manage My Details* page at any time, for updates.

**New application.** On the Home page, clicking My Application on the left menu, then clicking on the New Application box on the right, Applicants can access the page listing all the available Calls for Applications (*grant rounds*). Select within the *round 2020 Telethon Rare Diseases and COVID-19 Special Grant* the relevant call for the disease you are interested in and click **Apply** to create a new Application form.

Four calls are open focussing on different diseases/syndromes; you may **apply to one call only**.

## FILLING IN THE APPLICATION

The created Applications are listed in *My Applications* (link on the left hand menu of the Home page).

Applicants should pay careful attention to the **Guidelines and instructions**, as an Application failing to meet the requirements will be rejected. An accurate Application will facilitate the review process.

Use **English** language only. For abbreviations and acronyms not universally known, spell out the term the first time it is used, with the appropriate abbreviation in parentheses; the abbreviation should then be used thereafter.

The text must be single-spaced, not exceeding the character number limitations specified (which include spaces).

You can download a PDF of your Application at any time by clicking on the link *View/Print* at the Details page of your Application.

By clicking on *Save and Close* you can save and return to the Application form as often as you like.

**Required fields are indicated by red dots.** To successfully submit an Application, all required fields must be completed. Any required items missing before submission are listed in the *Validation* section.

When the Application is validated, the Applicant may *Submit* the Application, which is then automatically identified with the final Application number and displayed as *Under Review*. The Applicant will receive a confirmation email.

A submitted Application cannot be further modified; should you need to apply some amendments prior to the Call deadline date click the *Contact Us* on the left hand menu.

**Figures** - The Application forms include special upload fields dedicated to figures at the end of the Preliminary Results (not mandatory) and Scientific Strategy sections.

- Do not copy sections of already published papers.
- All figures and legends must be placed together in one PDF document in A4 format.
- In the Figures PDF, insert a footer with the name of the relevant section of the Application form followed by the indication “Figures” and the page number (for example a PDF uploaded into the Application section “Preliminary Results” should have the following footer: “Preliminary Results Figures - page 1 of 2”, “Preliminary Results Figures - page 2 of 2”, etc.)
- Important notice: in the PDF version of the Application, all Figures files will be automatically collected and displayed at the end of the Application form PDF as an Appendix. Make sure that the appropriate figure numbers are correctly indicated in the text.
- Please keep the PDF size below 25 MB. Use high resolution pictures only for photographs that require details; in this case a maximum resolution setting of 300 dpi (Photoshop: Image>Image Size>Resolution) for each photo is recommended.

- If you include charts or drawings in your PDF, a resolution of 100 dpi for each picture can be used.

**Make sure all the figures are perfectly readable both on the screen and printed.**

## APPLICATION SECTIONS

### *1. Project Overview*

#### *General Information*

##### **Project Title**

Insert the title of the project. **Please be aware that the title has to be filled in (and saved) by June 5, 2020. In case of title change you can modify it at any time before final submission.**

##### **Project duration**

Select the duration of the projects in months. The maximum duration of the present project allowed is 12 months.

##### **Type of Applicant**

Please select one of the following:

- New Applicant (never applied to any FT grant)
- Former Applicant (applied before to FT grants but never awarded)
- Former Grantee (applied before to FT grants and awarded)

##### **Type of Application**

Only *New Application* are allowed to this Call and this field is automatically prefilled.

##### **Applicant's Details**

General information about the Applicant are automatically collected from the Applicant's account

#### *Overview*

**Abstract** (max 2,000 characters spaces included)

**MeSH terms** (max 5)

Indicate up to five MeSH terms appropriate and specific for the proposed research

(<https://meshb.nlm.nih.gov/MeSHonDemand>).

**Added value and unmet need** (max 1,000 characters spaces included)

Explain how the proposed study will challenge current knowledge. Clearly state the current unmet need/s in the disease/syndrome that the proposed study will contribute to fulfil.

**Lay Summary – English** (max 250 words)

Summarize the project using an English lay language. This description is meant to serve for communication reasons and, as such, it may become public information. Therefore, do not include proprietary/confidential information.

### **Project Title – Italian**

Insert the title of the project in Italian.

### **Lay Summary – Italian** (max 250 words)

Summarize the project using an Italian lay language. This description is meant to serve for communication reasons and, as such, it may become public information. Therefore, do not include proprietary/confidential information.

## *Type of Research*

### **Disease Name**

Write the disease name.

### **Disease code**

Fill in all available disease codes: the **disease OMIM number** as given by the Online Mendelian Inheritance in Man (<http://www.ncbi.nlm.nih.gov/sites/entrez?db=OMIM>), the **ICD-10 code** as given by the International Classification of Diseases (<http://apps.who.int/classifications/icd10/browse/2010/en>) and the **Orpha Number** as given by Orphanet (<https://www.orpha.net/consor/cgi-bin/index.php?lng=EN>).

### **Research Type** (select all that apply)

Select the appropriate Research Type

### **Research Step**

Select the research step that best represents the proposed study:

1. genetic studies
2. studies of the mechanisms by which gene alterations cause the disease
3. studies of therapeutic approaches in cellular models
4. studies of therapeutic approaches in animal models
5. therapeutic clinical trials
6. diagnostic, observational and palliative clinical trials.

Should your project fall within more than one research step, select all you deem necessary to properly describe the study.

## *2. Scientific Strategy*

### *Background and Rationale*

#### **Background – Rationale – Objectives** (max 4,000 characters spaces included)

*Background:* 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 field. *Rationale:* State the hypotheses to be

tested and provide a realistic description of any expected scientific, technical and economic benefits. *Objectives:* Describe the overall objectives and what the specific research proposed is intended to accomplish.

**Preliminary Results** (max 4,000 characters spaces included)

Preliminary results **are not requested** for this call and therefore they are not mandatory.

Results are considered ‘preliminary’ only if unpublished. Published results, when deemed necessary, can be indexed as references.

**Background on Intellectual Property** (max 2,000 characters spaces included)

The Applicant is asked to indicate, to the best of her/his knowledge, any intellectual property where the Applicant is an inventor and any intellectual property owned by the Applicant or by a third party that would be necessary for the development of an approach to treat the disease/syndrome based on the results of the proposed project. For Applicant’s intellectual property, please indicate freedom to operate for such development.

**Preliminary Results Figures**

Refer to the “Figures” section (page 2 of this document) to create and upload the Figures’ PDF file (optional).

*Research Plan*

**Specific Aims and Experimental plan** (max 4,000 characters spaces included)

Provide a list of the specific aims explaining the scientific rationale. For each specific aim, provide an experimental plan by describing the general experimental design. Should new methodologies be developed or employed, please provide an appropriate description and state their advantages over existing methods.

The experimental plan must be based on an appropriate and accurate statistical design. State the potential difficulties and limitations of the proposed procedures and discuss alternative approaches to overcome them.

If the study involves vertebrate animals, please refer to the “Telethon rules and policy on animal experimentation” section (page 7 of this document).

Explain the need for collaborations (if any) to achieve the scientific aims of the proposed project. Include an explicit description of the collaborative elements that are essential for the project to be carried out. Any collaboration must be listed in the specific form (see page 9 of this document).

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

**Expected Outcomes and Future Development** (max 2,000 characters spaces included)

Describe the expected outcomes of the studies and the future project development (e.g., applications to grants/awards from corporations, foundations and government agencies; development of strategic partnerships).

**Timetable - GANNT Chart** (PDF to upload)

Please upload a GANNT chart describing the timeframe foreseen for the achievements of the Specific Aims.

#### **Experimental Plan Figures** (PDF to upload)

Refer to the “Figures” section (page 2 of this document) to create and upload the Figures’ PDF file.

#### *Cited Literature* (max 20,000 characters spaces included)

List all references. The list must include the name of all authors, year of publication, title, book or journal, volume number and page numbers. **Concise references are not allowed.**

### *3. Administrative Section*

#### **Host Institution and Overall Information**

Provide all the information requested. Please write the address in Italian (this address will be used for any postal deliveries addressed to you).

Download the Host Institution Agreement document, print it on the Institution’s letterhead, fill in the information and have it signed by the Institution’s Director or Responsible Official. The document must be provided in PDF format and uploaded within the Application. The original document should be kept by the Applicant for possible future requests by the Telethon Office.

NOTE: Applications with an incomplete Host Institution Agreement will be considered not compliant with the present Call and therefore will not be accepted.

#### **Applicant**

If the Applicant is not independent (*i.e.*, is not the Head of the Laboratory) an **independence statement must be uploaded in the dedicated box**. The independence statement-signed by the Lab Head-should contain the name of the Applicant, the Application Title and a clear statement by the Lab Head highlighting the Applicant’s capability to carry out the proposed experiments in due time.

Please insert the **name and email address** of the Lab Head in the corresponding field.

NOTE: Applications submitted by non-independent applicants lacking the independency statement will be considered not compliant with the present Call and therefore will not be accepted.

**Should the applicant hold other appointments (including foreign appointment)**, those have to be clearly indicated in “Any other Appointment” section and in the “*Host Institution Agreement*” document.

#### **Facilities and Resources**

Provide all the information requested and list all the key facilities available for implementing the project.

#### **Human subjects**

Indicate whether the study involves:

1. Human samples from a collaborator site or an external biobank

2. Human samples from individuals referred to the PI's Host Institution
3. Individuals enrolled in clinical trials
4. No human samples or subjects.

In cases 2 and/or 3, should the grant be approved for funding, funds will not be provided until the pertinent Ethics Committees' Approval 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>).

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

### **Vertebrate animals**

Specify whether or not experiments 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 rehomed. 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.**

## Budget

**Please note that the Budget section will appear on the left hand menu and can be filled in properly only once you insert the Host Institution name.**

Budget allocation does not need to be pre-specified in the application.

The amounts must be in Euro, please use whole numbers only.

The expenses report is required at project closure and only project-related expenses will be approved.

### *DIRECT COSTS*

The following expenses **are allowed** as long as they will be instrumental to achieve the project goals:

- Materials, Supplies, Services
- Salaries
- Travel costs for collaborations and learning purposes.

### *INDIRECT COSTS*

- Overhead (up to **5%** of the direct research cost)

The following expenses associated with the proposed research **are not allowed**:

- Equipments (including IT equipments)
- Full salaries for members of staff who already receive a regular wage
- Salaries, travel and/or housing related to sabbatical leaves
- Scientific Society memberships
- Organization of meetings and workshops
- Construction, alteration, maintenance, lab furnishing, rental of buildings or building spaces and utilities, fax and telephone costs
- Publication costs.



## Collaborations

The Applicant should list all the collaborators and the required information.

Once added, the collaborator/s will receive an Invitation email and, upon acceptance, they have to support the Application by sending a collaboration letter directly to the Applicant, written in English, which will be uploaded online by the Applicant (see also *TETRA Portal Instructions.pdf* document).

## Other Financial Support

It is important that the Applicant lists all the additional financial resources available in direct support of his/her research endeavors, including, but not limited to, research grants, cooperative agreements, contracts, and/or institutional awards.

Indicate:

- Title of the Project
- Status: Current/Pending. It is compulsory to indicate the relative Period
- Gross amount (in Euro)
- Granting agency
- Brief description of the project (max 2,000 characters spaces included)

If applicable, specify possible overlaps with the proposed project (max 1,000 characters spaces included)

## Personal Data and CV

**Education/Training:** begin with baccalaureate or other initial professional education, include postdoctoral training and residency training if applicable.

**Positions:** begin with pre-doctoral fellow.

**Personal Statement:** describe your research interest, achievements / contributions to science and future directions.

**Additional Info:** please use this box to add any additional information that you may consider relevant; (e.g.; honors, membership...).

**Financial interest disclosure:** Fondazione Telethon requires that Applicants declare all possible financial conflicts of interest that might be perceived as relevant (such as, for example, collaborations or direct involvement in profit or no-profit entities). However, these financial interests will not invalidate the Application, nor do they automatically disqualify it from being evaluated.

**ID Researcher Platform and Personal 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/>).

**Publications relevant to this Proposal-** Click on “*Add Publication*” to select up to 5 peer-reviewed publications from the list of publications already recorded in the Applicant’s account.

**Declaration**

The Applicant has to declare that the information included in the Application is accurate and complete and that he/she complies with Telethon's terms and conditions.

**SUBMITTING THE APPLICATION**

The deadline for **online submission is June 22, 2020 - at 5:00 PM (CEST)**.

Before the final submission, download the PDF of your Application to check all the sections; in particular check that all uploaded images are included in the PDF and are clearly readable. Please note that you are liable for the contents and quality of your Application in its final version.

Fondazione Telethon holds the responsibility and authority in making the final decision on the Application's completeness and eligibility.

After submitting the Application, a final Application number will be assigned to it. Please refer to this number in any future communications.

**May 25, 2020**

**FONDAZIONE TELETHON**