FONDAZIONE

TELETHON RESEARCH PROJECTS - 2017

GUIDELINES FOR PREPARING AND SUBMITTING THE APPLICATION ONLINE

Online Registration

Application forms for Telethon Research Proposals must be completed online at the following URL:

http://proposals.telethon.it/

If you are already registered, please enter your Login and Password.

In case of lost/forgotten Password, please enter your Login name and click on “Forgot your Password”: an automatic email containing your password will be sent to your email address.

In case of both Login and Password are lost/forgotten, please contact the Telethon Scientific Office at soffice@telethon.it. **Please avoid multiple registrations.**

If you do not have a registered profile yet, click on “**Registration**” and fill in all the fields; you will then receive a confirmation email containing Login and assigned Password, which allows access to the Application forms. Once you have logged in, you can change your Password using the “Change Password” function.

It is mandatory that the name inserted in the Registration Form corresponds to the Applicant’s.

Please note that your Login and Password will remain the same for future Calls.

General instructions for completing the Application

Applicants should pay careful attention to the 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 do not need to complete your Application in one session: remember to click on the "Update section" button each time to save your data before leaving the page.

The Full Application comprising the Core Project and the Supplementary Contents can be completed in any order. These are the sections of the Core Project and Supplementary Contents:

- **Core Project:**
  - General information
  - Overview
  - Scientific Approach
  - Cited Literature
- **Supplementary Contents:**
  - Cover Letter
  - Previous Achievements
  - Preliminary Results
  - Detailed Experimental Plan
  - Next Generation Sequencing and High Performance Computing
  - Personal Data and Curriculum Vitae
  - Personnel
  - Collaborations
  - Budget
  - Other Financial Support

- Host Institution
- Suggested Reviewers
- Notes
- Declaration and Privacy

You can download a PDF of your Application at any time by clicking on the link “Download PDF” at the bottom left of the page.

When completely satisfied with your proposal and ready for submission click on the "Update section" button, then on the "Send Application" button; a pop up window will prompt you to verify and confirm the following:

- I have uploaded the Host Institution Agreement
- I have filled in the Declaration and Privacy Statement section
- I have downloaded the project PDF and verified that all the figures are clearly legible and readable both in print and on computer monitors.

Once you confirm by pressing the button “Send”, the Application will then be formally closed by the system. An automatic message will be sent to you acknowledging that you have completed your Application and you will receive its PDF version.

Once you have submitted your proposal, you can modify your Application at any time, prior to the deadline date, by clicking on the "Edit Application" button. In order to save changes, remember to resubmit it again by clicking the "Submit Application" button. Only the last version submitted will be considered for evaluation.

Formatting editor - instructions

A text-formatting editor is available only in the specific fields inside the online form where “click to edit” is shown.

The main editor’s functions are the following:

- Copying and pasting text from Microsoft Word while retaining text formatting as well as tables
- Easy formatting of entered text with standard intuitive buttons
- Typing special characters including all Greek letters

The formatting editor allows the user to copy and paste text from Microsoft Word while retaining text formatting, with the following restrictions:

- You must use Microsoft **Word** to retain text formatting when copying and pasting: the use of other document editing software is not supported and could lead to errors in our online system
- In order to fully retain the original formatting in Word, use the button [**Paste from Word**], to copy text into the online field
- Once text is pasted from Word, the default font will be automatically set to Arial with minimum size of 16 pt (corresponding to Arial 11 when printed). Please verify in the PDF output that all text is clearly readable.

Hovering the mouse over the editor buttons will display a tooltip indicating their functions.

Please note that the font Symbol (Greek characters) is not supported: you should use the “**Insert Special Character**” button Ω in the formatting editor.

To verify that the correct text formatting has been applied check the PDF of the Application by clicking on the “Download PDF” button.

Figures

We strongly encourage the Applicant to limit the number of figures; too many unnecessary figures are not generally appreciated by reviewers. Do not copy sections of already published papers.

The Application forms include special upload fields dedicated to figures at the end of the Preliminary Results and Detailed Experimental Plan sections.

- All figures and legends must be placed together in one PDF document in A4 format.
- References to Figures should not be included in the Core Project (see "Core Project" section).
- Insert the name of the relevant section of the Application forms followed by the indication "Figures" and the page number in the page footer (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 under a section named "Figures". Make sure that the appropriate figure numbers are correctly indicated in the text.
- Please keep the PDF size below 25 MB, to avoid overloading our servers. 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 legible both on monitor and in print.

- To upload the "Figures" PDF file into the field, click the "Upload" button
- Click the "Sfoglia/Browse" button to select the PDF file from your computer
- Click the "Send File" button
- Click the "Close Window" button.

Core Project

This is the only part of the Application that will be made available to Reviewers during the Triage phase. Therefore, references to figures, preliminary data or other information reported in the Supplementary Contents should not be included here.

General Information

Project Title (max 150 characters) - In order to have full access to the Application forms you must insert the title of your proposed project. You can change it at any time, but only in this section. Please do not use all capital letters.

NOTE: only single-center proposals are admitted to the present Call for Applications.

Project duration - Indicate the duration of the project in years (min 1 - max 3 years).

Type of Applicant - Application – Choose the appropriate option according to the following descriptions:

- 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.
- 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.

Indicate the **Previous Application Number** in the specific box in case of a Revised or Renewal Application or for a New Application deriving from an **Exploratory Project** or from a **Multicenter Project**.

Indicate also the **Previous Role**, i.e. choose the appropriate option in the listed menu (Principal Investigator – Single Center; Coordinator – Multicenter; Partner – Multicenter).

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

Overview

Project Summary

Abstract (max 2,000 characters) – The Principal Investigator (PI) has to organise the Abstract into separate sections:

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

MeSH terms (max 250 characters) – Indicate up to five MeSH terms (http://www.nlm.nih.gov/mesh/meshhome.html) appropriate and specific for the proposed research .

Impact on patients (max 1,000 characters) - describe how close to therapeutic development, or to any other potential impact on patients, the proposed studies are.

Type of Research - Write the **disease name** and all its available 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** (if not available please indicate 'n.a.'), as given by the International Classification of Diseases (http://apps.who.int/classifications/icd10/browse/2010/en)
- the **Orpha Number** (if not available please indicate 'n.a.'), as given by Orphanet (http://www.orpha.net/orphacom/cahiers/docs/GB/List_of_rare_diseases_in_alphabetical_order.pdf);

If more than one disease is addressed, please separate names, OMIM numbers, ICD-10 codes and Orpha Numbers with semicolons.

Indicate the **research type**(s) (all that apply).

Select the **research step** that most truly represents the proposed activities:

1. Genetic studies to identify the genetic cause(s) of the disease
2. Studies of the mechanisms through 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.

If your project falls within more than one step, please choose the most relevant one; you may however select multiple steps if you deem it necessary to correctly describe your activities.

Scientific Approach

Central 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.

Include a very brief overview of your preliminary results, which should be further detailed in the Preliminary results section within the Supplementary Contents.

Specific Aims, Experimental Approaches and Expected Results (max 8,000 characters) - Describe the overall objectives and what the specific research proposed is intended to accomplish.

List the specific Aims of your project and briefly describe for each aim the proposed experimental approach to reach the stated objectives. An extensive description of the experimental approaches should be provided in the Detailed Experimental Plan in the Supplementary Contents.

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.

Relevance to Telethon (max 1,000 characters) - Clearly specify how the goals of the project fit with Fondazione Telethon's aims as declared in the mission statement (http://www.telethon.it/en/what-we-do/our-mission).

Please note that **diseases of proven genetic origin represent our focus**; in the proposed projects **the specific link to the genetic diseases under study needs to be clearly expressed and it will be specifically assessed by the Reviewers.**

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.

Supplementary Contents

This section, together with the Core Project, will be made available to the Reviewers only for those applications that are promoted to the Full Review evaluation phase following the triage.

Cover Letter

In case of a **Revised Application**, please fill in the Cover Letter form, specifying the previous application number.

Telethon Review Summary - The Telethon Scientific Office will upload the Telethon Review Report of the previous application in this section for those applications that will undergo full review.

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.

Previous Achievements (max 3,000 characters) - for former grantees only

In case of a **New** or a **Renewal Application**, the former grantee must briefly state the original goals and the scientific achievements of the previous, most recent, Telethon grant, listing the derived publications.

Unpublished results relevant to the current Application must be reported in the Preliminary Results section.

Preliminary Results (max 8,000 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 grant Application, as they aid the assessment of the likelihood of success of the proposed project.

Results are considered 'preliminary' only if unpublished.

Preliminary Results Figures - Refer to the "Figures" section (page 4 of this document) to create and upload the figures pdf file.

Detailed Experimental Plan

Detailed Experimental plan (max 12,000 characters) – Please restate the specific aims listed in the Core Project section avoiding as much redundancies as possible; thoroughly describe each aim explaining its logic by answering the following questions:

- What is the question being asked?
- How are you going to address it?
- What do you expect to find?
- What do you plan to do with those findings?

For each specific aim, provide an **experimental plan** by describing the general experimental design; if **new methodologies** are developed or employed, state their advantages over existing methods and provide a description.

In general, planning of experiments should 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. Discuss how data will be analysed and interpreted, and describe in detail the statistical methods to be employed.

If the study involves vertebrate animals, please refer to the "**Telethon rules and policy on animal experimentation**" section on page 13.

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 10).

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.

Feasibility, Pitfalls and Alternative Approaches (max 3,000 characters) – Please explain how the proposal is focused on achieving specific and feasible goals. In addition, state which pitfalls could arise during the research activity and which actions could be implemented to face them.

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

Clinical protocol **-** 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 experiences 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.agenziafarmaco.gov.it/it/content/normativa-di-riferimento-sperimentazione-clinica).

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 Fondazione Telethon in order for funds to be released.

In case of doubts, please contact the Telethon Scientific Office (soffice@telethon.it) before submitting the final application.

Experimental Plan Figures - Refer to the "Figures" section (page 4 of this document) to create and upload the figures' PDF file.

Next Generation Sequencing and High Performance Computing

Next Generation Sequencing [NGS]

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).

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

- Illumina (MiSeq, HiSeq, Genome Analyzer, etc.)
- Ion Torrent (PGM, Proton)
- Roche/454 (FLX+, Junior)
- SOLiD
- Third Generation/Single Molecule Sequencing

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

High Performance Computing [HPC] bioinformatics resources at Cineca

Fondazione Telethon partners with Cineca to offer grantees the possibility to exploit the HPC tools for the analysis of NGS data or to perform computer simulations of biological systems. HPC resources available at Cineca can be found at: http://www.hpc.cineca.it/content/hpc-science.

If the Applicant intends to use HPC resources, he/she is asked to provide information as follows:

For what purpose the HPC resources are requested? (max 500 characters) - specify the type of analysis, e.g. NGS analysis, molecular dynamic simulations, systems biology, docking, etc.

Systems already in use to run the application, if applicable (max 100 characters) - provide hardware specification of the system already in use.

Application of software packages (max 100 characters) - List each application or software package, including post-processing packages that are planned to be used. For each package, specify if it is open, proprietary or licensed and the communication and library requirements.

Estimated requirements

To fill in the following fields, please use this estimation for an experimental example as reference: "For the identification of sequence variants in a 100X human exome, 200 core hours total (parallel on 6 cores) and up to 32GB of memory are required."

Estimated number of core hours (max 100 characters) - to fill in this field the Applicant might use the experimental example to calculate: (elapsed time of a single run)* (number of cores used in a single run) * (total number of runs).

Estimated requirements for a typical run (number of nodes/cores, memory) (max 100 characters) - to fill in this field, please use as a reference guide the experimental example mentioned above, in "Estimated requirements".

Estimated storage requirements (max 100 characters) - provide an estimation assuming that the requirements for the experimental example (see "Estimated requirements") are: ~50 GB as input and ~170 GB as output.

In addition, please indicate if you are able to independently run your code or if you require a specialist support for using, installing or configuring new software or application packages.

For further details on the Cineca bioinformatics environment send an email to hpc-bioinformatics@cineca.it.

Personal Data and Curriculum Vitae

Personal data - Input your personal data avoiding all capital letters. Provide the telephone, fax and email address of your Office/Laboratory, as requested.

Unique Researcher identifying system - The broad adoption of a researcher identification standard is key to an effective management of research. Provide your personal author ID, e.g. ORCID (http://orcid.org/) or ResearcherID (http://www.researcherid.com) or Scopus author ID (http://www.scopus.com). If you do not have one, we suggest you to generate an ORCID ID.

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

Education and training (max 4,000 characters) - Organise information specifying date, place, institution, type of degree/diploma, and research field.

Employment and research experience (max 4,000 characters) - Organise information specifying period (from/to), place, institution/organization, type of employment, and field of interest.

Publications - Provide a list of up to 20 selected peer-reviewed publications and highlight with an asterisk (*) those relevant to the application.

Include the names 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" should be applied. **Concise references are not allowed**.

Personnel

Personnel (including the PI) are defined as, and should be limited to, key individuals whose contribution is deemed significant for the scientific development or execution of the project.

For each individual provide: name, birth date, degree, role in the project. Select from the dropdown menu the type of contract with the Host Institution (active or not), the annual percentage of effort in the project and whether a salary is being requested. If the Principal Investigator or any other Personnel are involved in more than one research project, make sure that the individual total effort is not exceeding 100%. Please note that **personnel to be recruited ("to be named") must be listed here and should be kept to a minimum**. For each person, the "role on the project" must be detailed. As an example, "molecular biologist performing mutational analysis" is appropriate, while "molecular biologist" is not sufficient.

Consultants should be included only when their level of involvement meets the previous definition. **An inadequately described role in the project and/or a mismatch with the annual effort, as also expressed in the budget, may result in the reduction of the budget approved.**

Collaborations

The PI should list all his/her national and/or international collaborations, specifying name, institution, whether the collaborator is related to the project and, if so, his/her relative contribution.

Collaborations must be supported by collaboration letters written in English (see the list of required documents below), which have to be uploaded in the online Application.

Budget

A maximum of 80,000 Euro/year is allowed; please note that a budget above 60,000 Euro/year will be scrutinized with particular attention.

The budget description must be accurate in all its parts and every item must be justified in the "Description/Justification" field and clearly related to the execution of the project. **Any omission, generic description, or miscalculation could lead to the project's rejection**.

All amounts must be expressed in Euro; please use **whole numbers** only.

For clinical studies: PIs are encouraged to contact the Telethon Scientific Office for assistance in defining the budget (soffice@telethon.it).

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

- Salary for the PI
- 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
- Major basic equipment such as incubators, hoods, -80°C freezers.

Direct costs

The following expenses associated with the proposed research are allowed:

Equipment - up to a total of **20,000 Euro** for minor essential equipment or a portion of a major piece of equipment. Each item must be clearly listed in the specific section and must be highly justified for the conduction of the proposed research.

IT equipment: The request for a personal computer should be clearly justified according to the research needs. The maximum amount allowed for IT equipment is 2,500 Euro and must be included in the "Equipment" section.

Materials, Supplies, Services - materials and supplies must be **listed by category**: glassware, chemicals, radioisotopes, etc. Services include items as animal housing (please provide the total number of animals and the cost per diem in the justification field), animal production (please specify if the service will be provided by a company), sequencing, peptide synthesis, biological material from biobanks (e.g. for TNGB refer to the cost recovery list http://biobanknetwork.telethon.it/Pages/View/pricelist), etc. Major cost items should be listed and properly justified.

Salaries - salaries for the project's staff (postgraduates, PhD students, junior/senior post-docs, technicians) holding a **temporary position** must be proportionate to the effort dedicated to the project (i.e. Full Time Equivalent as also specified under "Personnel"). Although not encouraged by Telethon, salaries for "to be named" people may be requested.

The names of Personnel for which a salary is requested must correspond to those listed under "Personnel".

Indicate the type of contract that will be applied and the level of seniority required. The salary requested should correspond to the level of seniority and to the annual effort declared. The amount must refer to the total employee cost (gross amount plus employment taxes).

Travel costs **-** travel costs for meetings/congresses (not more than 3,000 Euro annually).

Project-related travel costs must be carefully justified (destination, purpose and travel frequency) and adequately described in the project plan.

Costs allowed for travel are:

- transportation costs (train/plane/bus/taxi/car use, etc.)
- meals and lodging
- congress registration fee
- abstract submission fee.

Other expenses (each item should be detailed and justified):

- Allowed items: publication costs, reprints, journal subscriptions, books, sample and animal shipments. If software is requested, specify the necessity for the proposed research. Please detail the cost by item.
- Allowed items if overheads are not requested: repairing and maintenance of instruments, stationery, computer consumables (toner, external memory devices), mailing. Please detail the cost by item.

Indirect costs

Overheads - should be indicated up to 10% of the **direct research cost per year** and include for example: mailing, photocopying, office supplies, telephone expenses, equipment maintenance and repair, services such as radioactive waste and discarded solvent.

Please note that the percentage must not be calculated on the total budget requested but on the direct costs subtotal.

Based on the agreement between Fondazione Telethon and Consiglio Nazionale delle Ricerche (CNR), Applicants working at CNR laboratories should not include overheads in their budget.

Other Financial Support

It is absolutely mandatory that each Applicant lists in this section all 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 (max 250 characters)
- Status: Current/Pending. It is compulsory to indicate the relative period
- Gross amount
- Granting agency (max 250 characters)
- Brief description (max 2,000 characters)
- If applicable, specify possible overlaps with the proposed project (max 500 characters).

Host Institution

Host Institution - Provide all the information requested. The address must be provided in Italian, as it will be used for any postal deliveries addressed to you.

Print the completed Host Institution Agreement document on the Institution's headed paper and have it signed by the Institution's Director or Responsible Official. The document must be provided in PDF format and uploaded in the Application. The original document should be kept by the Applicant for possible future requests by the Telethon Office.

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

Applicant - If the PI is not the Chief of the Laboratory, the requested information should be exhaustively provided in the **Independence statement** field.

It is mandatory that **any foreign appointment** of the PI be clearly indicated in this section and in the "Host Institution Agreement" document.

Human subjects - Indicate whether the study involves:

1. Human samples from a collaborator site or an external biobank - fill in and upload attachment 1 (see "Additional Documents")
2. Human samples from individuals referred to the PI's Host Institution - fill in and upload attachment 2 (see "Additional Documents")
3. Individuals enrolled in clinical trials - send all relevant documentation (Ethics Committee's Approval, Informed Consent Form and Patient Information leaflet) to the Telethon Scientific Office (soffice@telethon.it) as soon as available
4. No human samples or subjects.

In cases **2** and **3**, if the grant is 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 (http://www.agenziafarmaco.gov.it/it/content/normativa-di-riferimento-sperimentazione-clinica).

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

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

Fondazione 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 116/92); moreover, approval by your Institutional 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 R**s (**R**eduction, **R**eplacement and **R**efinement, 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.

Documentation must be made available upon request.

Facilities and resources - List all the key facilities available for implementing the project.

Suggested Reviewers

The Applicant may suggest external referees - **not currently working in Italian Institutions** - expert in his/her 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 3 years should be avoided.

Fondazione Telethon reserves the right to choose external referees independently.

Notes (max 8,000 characters)

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.

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 automatically disregard any request of exclusion.

Declaration and Privacy

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.

Required documents to be uploaded

Clicking on the "**Required documents**" link on the left-hand menu bar online, the following documents: Host Institution Agreement and Human samples' Declarations (attachments 1 and 2) can be downloaded.

Submitting the Application

The deadline for **online submission is January 18th, 2017 -1:00 p.m**.

Before the final submission, download the PDF of your Application to check all the sections; in particular verify that all uploaded images are included in the PDF and are clearly legible.

Please note that you are liable for the contents and quality of your Application in its final version.

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

After sending the proposal, an automatic number will be assigned to it. Please refer to this number when requesting any further information or when sending hard copy documents.

November 15th, 2016 **FONDAZIONE TELETHON**