FONDAZIONE

TELETHON-UILDM CLINICAL PROJECTS - 2019

GUIDELINES FOR PREPARING AND SUBMITTING THE APPLICATION ONLINE

General Instructions

The Application form is available on the **TETRA - Telethon Projects Managements system portal** at https://projects.telethon.it.

Applicants are identified as **Lead Applicant** (in charge of creating and submitting the Application) and **Partner** (invited by the Lead Applicant, only for two-center Applications). For the **Partner invitation process**, refer to page 7 of this document.

Registration

To register, fill out and submit the Application, refer to the *TETRA Portal Instructions.pdf* - System Help available on the Home page.

Users of Telethon's discontinued grant management systems (http://proposals.telethon.it or http://webtric.telethon.it) must enter the **same email address used in their previous account** to be automatically recognized by TETRA. In case you don't remember that email address, please send an email to soffice@telethon.it. **Avoid duplication of accounts**.

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 and update your ***Basic Information*** and ***CV*** under the ***Manage My Details*** link on the left hand menu of the Home page. All this information will automatically populate the relevant fields of your Applications.

In the CV form ensure to update the following items: *Employment*, *Research Experience*, *Scientific Career*, and *Publications*, as all these are required for the submission of the Application. **You will not be able to edit this information directly from the Application form**; but you can return to the *Manage My Details* session at any time, for updates.

New Application

On the Home page under **New Grant Application**, clicking the **link *"here"*** Applicants can access the page listing all the available Calls for Applications (*grant rounds*). Click ***Apply*** to create a new Application form.

Completing 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).

The Full Application comprises the **Scientific Contents** and the **Administrative Section**, which can be completed in any order.

Application Forms

- **Scientific Contents:**
  - General information
  - Overview

- Cover Letter (for Revised Application only)
- Previous Achievements (for former Grantees only – New and Renewal Applications)
- Preliminary Results
- Clinical protocol and methods
- Cited Literature

- **Administrative Section:**
  - Administrative details for Lead Applicant and Partner(s)
    - Personal data and CV
    - Collaborations
    - Budgets and Personnel
    - Other Financial Support
    - Host Institution
  - Reviewers
  - Notes
  - Declaration

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.

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 Lead Applicant may ***Submit*** the Application, which is then automatically identified with the final Application number and displayed as *Under Review*. The Applicants 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

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 Scientific Approach 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).
- 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, 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.

Scientific Contents

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. Please do not use all capital letters.

Number of Centres **-** Indicate the number of centres participating in the study.

Project duration - Indicate the duration of the project (min 12 - max 36 months).

Type of Application **-** Choose the appropriate option among the list: *New*, *Renewal*, *Revised*.

Previous Application Number and ***Previous Role*** (where relevant, on the basis of the Type of Applicant and Application. Fill out the number of your previous Application and indicate your previous role by choosing the appropriate option from the listed menu (Principal Investigator - Single Center; Coordinator - Multicenter; Partner - Multicenter).

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

Methodological support and Pre-Submission inquiry

Telethon offers Applicants the assistance on methodological aspects related to the clinical protocol, a service provided by experts in medical statistics of the University of Milano - Bicocca.
Therefore, the Investigator wishing to explore the appropriateness of the design or the feasibility of a study that is still in a preliminary status may send a **"Pre-Submission inquiry**", filling out the form provided within the online Application.

In order to receive the above mentioned assistance, the Pre-Submission inquiry form available on the Telethon web site (http://projects.telethon.it) must be filled out in Italian and submitted via email to the Telethon Scientific Office together with the documentation requested within the Pre-submission inquiry form no later than **January 21st, 2019.** Requests that are incomplete or submitted after this date will not be considered (although this has no bearing on the submission of the final version of the proposal).

Written feedback by the consultants will be sent to the Investigators by **February 28th, 2019**.

Please note that any support received does not guarantee the success of the Application.

Overview

Abstract (max 2,000 characters) – Organise the Abstract providing the following information:

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

Coordination and Management - Multicentre Studies only (max 4,000 chars) - The Lead Applicant should specify in this section how the multicentre project will be managed, indicating strategies aimed at:

- monitoring activities of all centres
- facilitating communication
- promoting exchange of ideas and methodological approach
- stimulating the analysis and the integration of results.

Role and contribution of partner(s) in the project - Multicentre Studies only (max 4,000 chars) - The Lead Applicant is asked to describe the contribution of all Partners, explain why each of them is necessary to the success of the project, clarify the complementarities of approaches that justifies their participation and to highlight how the synergy among them will produce greater results over the sum of individual contributions.

Relevance to Telethon *(max 1,000 chars)* - Clearly specify how the goals of the project fit with the Fondazione Telethon's and UILDM's aim of improving the quality of life of patients affected by genetic muscle disorders. Proposals targeting other diseases, although of proven genetic origin, will not be processed for review.

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

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.

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)** (as many as necessary).

Cover Letter (max 15,000 chars)

The Cover Letter section is accessible only for **Revised Applications.**

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

Cover Letter (max 15,000 characters) - 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 - for former Grantees only, in case of a New or a Renewal Application

Provide the **Project number and title of the most recent Telethon grant** (max 350 characters); and briefly state the original goals and the scientific **achievements**, also listing the derived publications (max 3,000 characters). Unpublished results relevant to the current Application must be reported in the Preliminary Results section.

Scientific Strategy (max 20,000 chars)

Background - Explain the impact of the problem addressed by the proposed project. Critically evaluate the existing knowledge and identify the specific gaps 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 in the Application is intended to accomplish. The objectives of the study must be logical, feasible and innovative; they must represent a significant step forward beyond the current state of the art and include substantial original work.

Scientific Strategy Figures - Refer to the "Figures" section (page 3 of this document) to create and upload the figures' pdf file.

Preliminary Results (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. For Multicentre Studies, preliminary results of the Centres involved in the study should be listed site by site, if applicable.

Results are considered 'preliminary' only if unpublished.

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

Clinical protocol (max 30,000 chars)

Clearly define:

1) Study design, i.e. blind, double blind, open, etc.

2) Study population, i.e. number of patients based on power calculation, 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.

The Clinical project must be completed with the 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 a Clinical study has already been defined, the clinical protocol has to be uploaded in this section. Otherwise, if the study is funded, the Telethon Scientific Office will ask for the protocol and related documents before releasing any funds dedicated to the clinical study.

Explain the need for collaboration (if any) to achieve the scientific aims of the proposed project. Indicate how the idea of collaborating originated, the different approaches, which each collaborator will bring to the overall study, and how the collaboration will be conducted. Any collaboration must be listed in the specific form (page 7 of this document).

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.

Timetable (max 4,000 chars) - Provide a tentative sequence or timetable for the project.

Methods (max 8,000 chars) - Describe any new laboratory procedure or new methodology employed in the study and state the advantages over existing methods.

Clinical Protocol Figures/Documents - Refer to the "Figures" section (page 3 of this document) to create and upload the figures pdf file.

Cited literature (max 20,000 chars)

List all references accordingly. The list must include the names of all authors, year of publication, title, book or journal, volume number and page numbers. If bibliographic management software is being used, the format of the journal "Developmental Dynamics" may be applied. **Concise references are not allowed**.

Administrative Section

Administrative Details

The **Administrative Details main page** displays three Summary tables with information on: *Lead Applicant & Partner Organisation(s)*, *Contacts* and *Total Budget* details.

Multicenter projects - From this page, the Lead Applicant invites the Partner(s) to join the Application.

Partner invitation

To enlist and invite any Partner, the Lead Applicant will perform the following steps (if needed refer to the *TETRA Portal Instructions.pdf* System Help):

1. Add Grant Organisation – If the Partner's Organisation is not already available in the IntelliSence menu, add the new one and save it. The newly added Organisation will be displayed in the overview table.

2. Add Participant: follow the flow chart. Select your Partner *Grant Organisation******; Next >> *Select* the *Contact*, if available; if not, Next >> *Contact Search*: type the email address, click on *Search*, if available click on *Select*, if not available > *Add New Contact* – fill out the required fields and *Add Contact* > *Contact Notification*: the Invitation email is displayed, *Confirm* that you wish to send this message (check the box) and *Send the Invitation*.

Note: because of technical issues you cannot select a Partner belonging to your same organisation or institution. If this is the case, please register your Partner organisation as New Organisation, adding the department name *(for instance Organisation, Department).

Partner confirmation

If a Researcher is invited to participate in an Application as Partner, he/she will be informed by email. Clicking on the link in the invitation email opens a page where he/she can *Accept* or *Decline* this invitation.

In order to make a decision, the invited Partner may access the related Application in the ***My Co-Applications*** left-hand menu and examine the Application's details. Once decided, click *Confirm* or *Reject*, as appropriate.

Once the Partner has accepted the Invitation, the Partner will be able to register with the system and to edit the Application form.

From within the **Lead Applicant & Partner Organisation(s) table**, click on the **EDIT link beside the Organisation(s) in order to have access to the Administrative Details sub-menu**, consisting of the following sections: *Personal Data and CV, Collaborations, Budget and Personnel, Other Financial Support, Host Institution.* These Sections have **to be completed separately by both the Lead Applicant and the Partner.**

Personal Data and Curriculum Vitae

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

Selected Publications - Click on *"Add Publication"* to select up to 20 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 Researcher Platform and 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.

Collaborations

The Applicant should list all his/her national and/or international collaborations with the required information.

Actively involved collaborators are those directly related to the project and, as such, their contribution to the project must be described in the dedicated field. Once selected, the actively involved collaborators receive an Invitation email and, upon acceptance, they **must support the Application by sending collaboration letters, written in English, which have to be uploaded in the online Application by the Applicant**.

Budget and Personnel

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.

Personnel (including the Lead Applicant and Partner) are defined as, and should be limited to, key individuals whose contribution is deemed significant for the scientific development or execution of the project. Please note that **personnel to be recruited ("to be named") must be listed here and should be kept to a minimum**.

For clinical projects that entail the enrolment of a number of patients necessary for power calculation it is advisable to identify start-up and other fixed costs separately from patient-related costs (variable costs). Fixed costs are incurred regardless of the number of subjects enrolled, while variable costs are strictly related to the expected number of patients. Full reimbursement of variable costs will be dependent on the actual number of enrolled subjects. The PI may contact the Telethon Scientific Office for assistance (soffice@telethon.it) if deemed necessary.

To **ADD** an Item click on the relative button and fill out the required information.

Direct costs

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

- Salary for the Lead Applicant/Partner
- 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.

The following expenses associated with the proposed research **are allowed**:

Coordination costs **-** Allowed only for the Lead Applicant of a Multicentre project which includes at least 3 centres. This amount should be requested up to a maximum of 5,000 Euro per year and is intended to cover coordination administrative costs, which need to be well specified.

Start-up costs

- Database set-up
- Ethics Committee fees
- Medical supplies
- Pharmacy set-up costs
- Printing of documents (i.e. CRF, informed consent)

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 conduct 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**: consumables, antibodies, reagents, 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.

Personnel and Salaries - 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.**

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). Although not encouraged by Telethon, salaries for "to be named" people may be requested. 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).

If a salary is not required, enter 0 in the Salary field.

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.

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

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.

Other Financial Support

It is 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.

Click on the **Add** button and Indicate:

- Granting agency (max 250 characters)
- Title of the Project (max 250 characters)
- Status: Current/Pending. If *current*, it is compulsory to indicate the relative period (Start - End date)
- Gross amount, Currency
- Brief description (max 1,000 characters)
- If applicable, specify possible overlaps with the proposed project (max 500 characters).

Host Institution

Host Institution **-** Download the HI Agreement document, print it on the Institution's headed paper, fill out the information 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 with an incomplete Host Institution Agreement will be considered not compliant with the present Call and therefore will not be accepted.

Applicant - Provide all the information requested.

If the Applicant is not the Chief of the Laboratory, the **Independence statement** must be completed (max 1,000 characters).

It is mandatory that **any foreign appointment** of the Applicant be clearly indicated in this 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 - download, fill out and upload Attachment 1
2. Human samples from individuals referred to the PI's Host Institution - download, fill out and upload Attachment 2
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 staff (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.aifa.gov.it/content/modulistica-sperimentazione-clinica).

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

Suggested Reviewers

Suggested Reviewers **-** 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 3 years should be avoided.

Telethon reserves the right to choose external referees independently.

Excluded Reviewers **-** 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 (max 8,000 chars)

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

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 March 31st, 2019 at 1:00 p.m**.

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

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 related to it.

December 12th, 2018 **FONDAZIONE TELETHON**