



COMITATO TELETHON FONDAZIONE ONLUS

GUIDELINES FOR PREPARING AND SUBMITTING THE APPLICATION ONLINE

TELETHON–UIIDM CLINICAL GRANTS – CALL 2009

Introduction

Application Forms for Telethon Grant Proposals must be completed online at the following URL:
<http://proposals.telethon.it>

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

If you have forgotten just your Password, please enter your Login name and click on “Forgot your Password”: you will then receive an automatic email with your password.

If you have forgotten both the Login and Password, please contact the Telethon Scientific Office at soffice@telethon.it. **Please do not make multiple registrations with the same name.**

If you do not yet have a registered profile, click on “Registration” and fill out all the fields; you will then receive an email confirming your Login and assigned Password that will give you access to the Application forms. We encourage you to choose a Login that you can easily remember.

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. Once you have logged in, you can change your Password using the function “Change Password”.

Applicants should pay careful attention to the instructions, because an application that fails to meet the requirements will be rejected. An accurate application will facilitate the review process.

In the agreement with experts in medical statistics of the University of Milano – Bicocca, Telethon offers the Principal Investigator assistance on methodological aspects related to clinical protocols.

The Principal Investigator may make use of this service if he/she deems it necessary.

Investigators wishing to explore the appropriateness of the design or the feasibility of a study that is still in a preliminary status may submit a “Pre-Submission inquiry”. The requests received will be evaluated together with members of the UIIDM Scientific Committee, who will offer support to develop those projects deemed more relevant and worthwhile to pursue, according to the objectives of this call.

In order to receive either of the above mentioned assistance, the Pre-Submission inquiry form (available on the Telethon web site <http://proposals.telethon.it>) must be filled out in Italian specifying the type of consultancy needed as requested and submitted via email to the Telethon Scientific Office, no later than April 27, 2009. Requests 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 May 25, 2009. Please note that any support received does not guarantee the success of the application.

The Application must be submitted electronically once completed.

INSTRUCTIONS FOR COMPLETING THE APPLICATION FORMS

- The Application Forms are organized into sections, which can be completed in any order.
- You do not need to complete your Application Form in one session: remember to click on the "Update section" button to save your data before leaving the page.
- When you are completely satisfied with your proposal click on the "Update section" button, then on the "Send Application" button; the form 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, only prior to the deadline date, clicking on "Retrieve Sent Application" button: remember to resubmit it again with the "Send Application" button.

Telethon-UILDM Application - General Instructions

Read and follow the instructions carefully.

Use English language only, except for the lay summary, which must be both in Italian and in English. For terms 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)**.

If working online, please always remember to click the "Update Section" button in order to save the data inserted in the forms.

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

Telethon-UILDM Application - Specific Instructions

General Information

Project Title (max 250 chars) - In order to have full access to the Application Forms you must write the title of your proposed project. You can change it at any time, but only in this section.

Centres involved in the study - Indicate whether the project involves a **single centre** or if it is a **multicentre** effort; in the latter case, please specify the total number of participating sites. **The number of sites can be modified at any time: if the number is reduced, the system will automatically remove the last partner and all her/his data.**

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

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

A **New Applicant** is a researcher who has never applied to a Telethon call, a **Former Applicant** is a researcher who has already applied to a Telethon call but has never been funded, whereas a **Former Grantee** has been already funded by Telethon in the past.

New Applicants may propose a **New Application** only.

Former Applicants may propose a **New Application** or a **Revised Application**.

Former Grantees may propose all the above types of applications or a **Renewal** of a previously funded project.

Applicants proposing a **Revised Application** must fill out the Cover Letter Form.

Study classification - The Applicant has to indicate the kind of study proposed. "**Clinical Study**" applies to all studies that foresee enrolment of a number of patients necessary for power calculation, whereas "**General Research Study**" includes projects focused on preclinical investigation or on analyses that do not directly involve patient recruitment. For Clinical Studies, the total number of patients expected to be enrolled in the trial should be reported.

Scientific Report of the previous Telethon-UILDM project – This section has to be filled out only by Former Grantees, for the most recently completed Telethon-UILDM clinical grant. Indicate whether and when the Final Report has been submitted. If not already sent, the scientific report has to be enclosed in the specific section ("Research Plan - Scientific Report").

Research Plan

This part should include all information pertinent to the evaluation of the clinical project.

For Multicentre Projects, the Coordinator should discuss all issues of the study as a whole, highlighting both the advantage of the synergy among all centres and the individual contributions, where appropriate.

Project Overview

Abstract (max 2.000 chars) – The Applicant (Principal Investigator/Coordinator) has to organise the Abstract into separate sections:

- Objectives
- Background/Rationale
- Description of the project
- Anticipated output

For Multicentre Studies only:

Role and contribution of partner(s) in the project (max 4.000 chars) - The Coordinator is asked to fill out this part, listing the specific contribution to the project of each partner.

Coordination and Management (max 4.000 chars) – The Coordinator 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

Keywords (max 250 chars) - Indicate a maximum of five appropriate keywords, which represent the contents of the research.

Relevance for Telethon (max 1000 chars) - Clearly specify how the goals of the project fit with the Comitato Telethon's and UILDM's aim of **improving the quality of life of patients affected by genetic muscle and nerve disorders, and spinal muscular atrophy (SMA)**. **Proposals targeting other diseases, although of proven genetic origin, will not be processed for review.**

Therapeutic potential - (max 1000 chars) - Potential progress towards therapeutic solutions should be specifically addressed.

Type of Research - Please write the disease name and number (if any) given by the Online Mendelian Inheritance in Man (<http://www.ncbi.nlm.nih.gov/sites/entrez?db=omim>). Indicate the research type(s) (as many as necessary).

Lay Summary – This description is meant to serve as a succinct and accurate description of the proposed work when separated from the application. If the application is funded, this description, as it is, will become public information. Therefore, do not include proprietary/confidential information.

The Applicant is asked to summarize the project (both in Italian and English) using a lay language, specifying the relevance to Telethon's mission and any progress envisaged towards the cure of the disease.

Figures and Tables – Create and save a .jpg file formatted as a single A4 page where you may paste your images, graphs or tables (Photoshop users: please save the .jpg file in RGB color mode only). Please indicate in the file the Principal Investigator's/Coordinator's name.

Only if your images do not fit on a single A4 page, you can attach a file in .pdf or .rtf format; please note that in this case the pictures will not automatically be displayed in the application form but a separate link to download the file will appear in its place.

Cover Letter (max 15.000 chars)

In the case of a Revised Telethon-UILDm Application, please fill in the Cover Letter Form. Please specify the previously assigned application number.

Telethon Review Summary – The Telethon Scientific Office will upload the Telethon Review Summary of the previous application in this section.

Cover Letter - When applying with a Revised Application that was excluded by triage the Cover Letter Form must be provided highlighting modifications.

When applying with a Revised Application that received written comments the Cover Letter Form must also include a detailed reply to the critiques.

Scientific Report of the Previous Telethon-UILDm Project (for Former Grantees only)

Please note that, in the case of a **Renewal Application** or of a **New Application by a Former Telethon-UILDm Clinical Project Grantee**, a scientific and an administrative reports are required for the completed Telethon-UILDm clinical grant, if not already submitted.

Scientific Report - Provide all the requested information, including the title of the completed project, the original abstract, as it appeared in the application, a summary of the project's achievements, the contribution of each partner (for multicentre projects only). Please also state any obstacle met during the research and/or any divergence from the original plan. Include a list of the resulting publications and the submitted manuscripts.

Administrative Report – Please click the link “Additional Documents” and download the attached forms ***Dichiarazione costi sostenuti.xls*** and ***Riepilogo centri.xls*** (for Multicentre Coordinators only) - and fill it/them out according to the instructions provided ***Istruzioni Rendiconti Amministrativi.pdf***. Finally, send, by email, the completed form(s) to the address reportamministrativi@telethon.it **by July 15, 2009**.

The administrative report must prove that a minimum of 30% of the last year's funds has been spent.

Coordinators of a former Multicentre project are responsible for sending both their own administrative report and the summary of the Partners' centres reports.

Partners are not required to send the administrative reports of their own previously funded projects.

For single-centre projects, the administrative report is not required if the funds are directly managed by Telethon (*Gestione Diretta*).

NOTE: If the Administrative Report is required, sending it by email within the deadline is mandatory, otherwise the Application will not be processed for review.
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Background (max 8.000 chars)

Describe the background of the proposed project, evaluate critically the existing knowledge and identify the specific gaps that the project is supposed to fill. State concisely the importance and health relevance of the research described in this application by relating the specific aims to the broad, long-term objectives.

Specific aims and rationale (max 16.000 chars)

List the objectives of the study and what the specific research proposed in this application is intended to accomplish. Provide clear and concise information about the primary endpoint(s) of the proposed research and give a realistic description of any expected scientific, technical and economic benefits. The objectives of the proposal must be convincing and feasible, and must be innovative, i.e. they must represent a significant step forward beyond the current state of the art and include substantial original work.

Preliminary results (max 16.000 chars)

Provide an account of preliminary studies pertinent to the proposed research. This information will also help to establish the experience and competence of the Investigator to pursue the proposed project. Peer review committees generally view preliminary data as an essential part of a research grant application, as they often 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.

Clinical protocol and methods

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. If new methodologies are developed or employed state their advantages over existing methods. 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. Describe alternative treatments and procedures (where appropriate) that might be advantageous to the subjects.
- 6) Data management and statistical plan. Discuss how data will be collected, analyzed and interpreted. Describe in detail statistical methods.
- 7) Provide a sequence or timetable for the project.

Indicate the sources of research material obtained from individually identifiable **living human subjects** in the form of specimens, records, or data. State whether the material or data will be obtained specifically for research purposes or whether use will be made of existing specimens, records, or data.

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. 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.

The Clinical project must be completed with Ethics Committee's Authorisation in accordance with the laws of the Italian Ministero della Salute (<https://oss-sper-clin.agenziafarmaco.it/normativa.htm>).

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.

Cited literature (max 16.000 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.**

Personnel

Personnel (including Principal Investigator/Coordinator/Partner) are defined as, and should be limited to, key individuals who contribute in a substantial way to the scientific development or execution of the project.

For each individual provide: name, role on the project, degree, date of birth and the annual percentage of effort in the project. If a salary is being requested, indicate if the person already works at the Host Institution and report the type of contract (permanent position or temporary position. For temporary position specify in Italian the type of contract). **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 specified in detail. For example "technician" is not satisfactory; "technician for quality control and data entry" is appropriate. Consultants should be included only when their level of involvement meets the previous definition.

Proposed Budget

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

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.

For Multicentre proposals: each partner is asked to fill out details about her/his budget in the corresponding boxes. The system will automatically create a summary section with the total budget.

Applicants have to fill out only those items that better apply to the project.

For clinical projects that foresee the enrolment of a number of patients necessary for power calculation it is advisable to identify startup and other fixed costs separately from patient-related costs (variable costs). Fixed costs are incurrent 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 Principal Investigator/Coordinator may contact the Telethon Scientific Office for assistance (soffice@telethon.it).

DIRECT COSTS

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

Startup costs

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

Patient-related costs (variable costs: to be filled out only for a clinical study, in which the exact number of patients is defined)

- Lab work, biochemical assays, etc.
- Mailing of drug and/or specimens
- Salary for personnel dedicated to patients (examining researcher, secretary, etc.)
- Pharmacy and drug administration
- Physical exams and medical assessments
- Reimbursement of travel expenses to patients
- Subject screening

Total patient-related costs

At the bottom of the online "Proposed Budget" section, each partner has to report the total number of patients and the total patient-related costs of his/her centre. The cost per patient calculation will be available in the project .pdf or in the read-only mode.

Equipment

Up to a total of **20.000 Euro** for minor essential equipment or a portion of a major 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 (i.e.: personal computer, printer, scanner, etc.): must be included in the Equipment section only. *The need for a laptop instead of a PC should be well explained according to the research needs*, and must not represent a benefit for the Researcher (i.e. justification as "for meeting presentation" are not acceptable). The amount allowed for IT equipment is up to a total of 2.500 Euro (for each Partner).

Therefore if IT equipment is requested, the total maximum amount for Equipment allowed is 22.500€.

Materials, Supplies and Services

Materials and supplies must be listed by category: glassware, chemicals, radioisotopes, etc. Services include items as *Sequencing, Peptide Synthesis, etc.*

Salaries

Please report here the salary for Personnel not dedicated to patients (i.e. Clinical monitor, data manager, statistician, consultant fees, etc.) or for other project staff (post-docs, technicians, etc.) holding a temporary position.

Specify names, role on the project of the recipients, and amount under the specific section. **These data must correspond to those reported in the "Research Plan-Personnel" section.** Although not encouraged by Telethon, salaries for "to be named" people may be requested.

Travel costs: in the description field please split travel costs into

- **Travel costs for congresses** (not more than **3.000 Euro** annually)
- **Project-related travel costs of personnel** (which must be carefully justified: destination, purpose and travel frequency – and adequately described in the project plan)

Please note that the following costs are allowed:

- *Transportation Costs* (train/plane/bus/taxi/car use...);
- *Boarding and Lodging*;
- *Congress Registration Fee*;
- *Abstract Submission Fee*.

Other costs (should be detailed):

- **Allowed items:** publication costs, reprints, journal subscriptions, books. If software is requested, specify that needed for the proposed research
- **Allowed items, if overhead are not requested:** stationery (paper etc.), computer consumables (Toner, CDs, etc.), repairing & maintenance of instruments, postage costs.

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

- Major basic equipment such as incubators, hoods, -80°C freezers
- Salaries for Principal Investigator/Coordinator/Partner
- Salaries for members of staff who already receive a regular payment unless they are patient related
- Salaries, travel and/or housing related to sabbatical leaves
- Scientific Society memberships
- Organization of meetings and workshops
- Construction, alteration, maintenance, lab furnishing or rental of buildings or building spaces
- Fax and telephone costs

INDIRECT COSTS

Overhead should be indicated up to 10% of the **direct cost per year**, and include equipment maintenance, postage costs, photocopying, office supplies, telephone and fax expenses.

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

Centre's patient-related costs sum up

Each partner has to report the total number of patients and the total patient-related costs of his/her centre. The cost per patient calculation will be available in the project .pdf or in the read-only mode.

Applicant

The Principal Investigator/Coordinator/Partner must input his/her data as requested.

Personal Data and Curriculum Vitae

Personal data - Input your personal data. Please provide your telephone, fax and email address of your Office/Laboratory.

Financial interests disclosure - Telethon invites applicants to highlight 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 4000 chars) - Organise information specifying date, place, institution, type of degree/diploma, and research field.

Employment and research experience (max 4000 chars) - Organise information specifying period (from/to), place, institution/organization, type of employment, and field of interest.

Publications (max 8000 chars) - **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.**

Provide **three** separate lists of up to **5** publications each, corresponding to:

- your most recent peer-reviewed publications
- your top peer-reviewed publications in your career
- your most relevant peer-reviewed publications related to the proposed research project.

NOTE: the same publications may be included in more than one list.

Host Institution

Provide all the information requested clearly and in detail. **Please write the address in Italian. Please note that this address will be used for any postal deliveries addressed to you.**

Please 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 then be scanned, saved in .PDF format and uploaded into the application.

Please find below some general scanner settings for written documents:

- Resolution: 100 dpi
- Colors: 256 shades of grey
- Image size: A4
- Final jpeg file compression: medium

NOTE: It is mandatory to upload the Host Institution Agreement, otherwise the Application will not be processed for review.

Human subjects - Indicate whether the study involves:

1. human samples from a collaborator site or an external biobank – fill out and upload attachment 1 (see “Related documents”)
2. human samples from individuals referred to the Principal Investigator's/Coordinator's Host Institution – fill out and upload attachment 2 (see “Related documents”)
3. individuals enrolled in clinical trials – all relevant documentation (Ethics Committees' approval, Informed Consent Form and Patient Information leaflet) has to be sent to the Telethon Scientific Office – Via Nino Bixio, 30 20121 Milano as soon as available.

Please note that in cases **2** and **3**, if the grant is approved for funding, funds WILL NOT BE AWARDED until the pertinent Ethics Committee's Approval has been obtained. Please activate in due time all necessary procedures to obtain this approval in accordance with the relevant Italian laws (<https://oss-sper-clin.agenziafarmaco.it/normativa.htm>)

You can find an example of Informed Consent Form plus Patient Information leaflet at http://www.telethon.it/sites/researchers/SiteCollectionDocuments/consinf_23_01_2004.pdf.

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.

The Comitato Telethon Fondazione Onlus will not support experiments on animals unless there is no alternative. However, the Comitato Telethon Fondazione Onlus recognises that they are often necessary in many areas of biomedical research and appreciates the importance of the refinement of procedures aimed at minimising pain or distress caused to laboratory animals.

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

Other Financial Support

It is absolutely mandatory that the Principal Investigator/Coordinator/Partner list in this section all financial resources available in direct support of his/her research endeavours, including, but not limited to, research grants, cooperative agreements, contracts, and/or institutional awards.

Indicate:

- Title of the Project (max 250 chars)
- Status: Current/Pending. It is compulsory to indicate the relative period
- gross amount
- Granting agency (max 250 chars)
- Brief description (max 2000 chars)
- If applicable, specify possible overlaps with the proposed project (max 500 chars)

Collaborations

The Principal Investigator/Coordinator/Partner should list in her/his dedicated section all the national and/or international collaborations, specifying name, institution, whether the collaborator is related to the project and, if so, the relative contribution. Please note that

collaboration letters must be written in English (see Additional Documents list) and must then be scanned, saved in .pdf format and uploaded into the application.

Please find below some general scanner settings for written documents:

- Resolution: 100 dpi
- Colors: 256 shades of grey
- Image size: A4
- Final jpeg file compression: medium

Suggested Reviewers

The Applicant may suggest external referees - **not currently working in Italian Institutions** - in her/his fields of research, who could competently review her/his application. Please do not list individuals who have been associated with the Applicant within the last 3 years.

Should you prefer to exclude direct competitors, please indicate their names in the NOTES.

The Comitato Telethon reserves the right to choose external referees independently.

Notes (max 8.000 chars)

Write here personal comments, details or additional information you wish to add to any specific sections of the application form. Please indicate which section you are referring to and the reasons for including more information.

Declaration and Privacy Statement

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).

PLEASE NOTE: If the Declaration and Privacy Statement is not filled out by the Applicant, the Application will not be processed for review.

Additional documents to be uploaded

Additional documents to be uploaded into the proper Application's sections or emailed can be downloaded by clicking on the link "**Additional documents**" on the left-hand menu bar online.

Submitting the application

The deadline for **online submission** is **July 15, 2009, 12.00 (midday)**.

You can modify your application until the deadline. Click on the "Send Application" button to submit your completed application. An automatic electronic system will verify whether all fields have been filled out and notify you if any still have to be completed.

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

The **Administrative Report** must be sent to the email address reportamministrativi@telethon.it by **July 15, 2009**.

A HARD COPY OF THE ONLINE APPLICATION DOES NOT NEED TO BE SENT.

March 25, 2009

COMITATO TELETHON FONDAZIONE ONLUS