



TELETHON-UILDM CLINICAL PROJECTS

CALL FOR APPLICATIONS - 2015

General

Fondazione Telethon will devote funds deriving from the UILDM (*Unione Italiana Lotta alla Distrofia Muscolare*) fund-raising campaign towards **clinical research projects aimed at improving the quality of life of patients affected by genetic muscle and nerve disorders, or by spinal muscular atrophy. Being muscular dystrophies and spinal muscular atrophy the main pathologies addressed by the UILDM, proposals on these diseases will be considered of highest relevance.**

Multicentre and multidisciplinary projects are encouraged.

Clinical research projects in cardiology, gastroenterology, internal medicine, neurology, orthopaedics, psychology and respiratory medicine, aimed at developing preventive, diagnostic, therapeutic and rehabilitative aspects of the above listed disorders are admitted.

In particular, the research goals are:

- Controlled clinical studies for the evaluation of pharmacological and rehabilitative therapies
- Cost-benefit analysis of models for assistance outside the hospital setting
- Creation of data banks
- Development and application of new techniques and technologies aimed at improving expectoration, speech, mobility and posture
- Development of new diagnostic and therapeutic techniques for cardiology and respiratory medicine settings
- Development of protocols for evaluating muscular force and endurance
- Evaluation and treatment of cognitive and neuropsychological complications
- Evaluation and treatment of orthodontic defects and disorders in swallowing and digestive transit
- Genetic, clinical and instrumental approaches in prenatal and neonatal diagnosis
- Models for training of medical and paramedical personnel and support for caregivers
- Nutritional evaluation in relation to patients' metabolic status and body type
- Optimisation of ventilatory support in the treatment of acute and chronic respiratory insufficiency
- Prevention and treatment of limb and spinal column deformities.

Eligibility

Eligible Host Institutions

Only research proposals submitted by Investigators working either in public or private **Italian non-profit research Institutions** are eligible.

Investigators belonging to the Telethon Institutes (TIGEM, TIGET and DTI) can apply as Partner only. In the case of TIGEM or TIGET Investigators, however, no budget can be requested for their participation in the project.

Eligible applicants

In order to be eligible for funding, all Applicants (i.e.: the Principal Investigator – PI – of a single-centre project, or the Coordinator and Partners of a multicentre project) must prove to be:

- scientifically independent
- resident in Italy.

If the Applicant does not hold a permanent position, the Host Institution should be aware that the salary of the Investigator cannot be requested within the Application as part of the Telethon-UILDM Grant.

A dual appointment in a foreign Institution must be notified in the Application and is allowed only if it does not conflict with the effective conduct of the Telethon research project. In any case, it is mandatory that the budget is used for research to be carried out at the Italian Host Institution only.

If the Application is successful but the scientific independence of the Applicant is not clearly evident to the Scientific Committee, the Committee may recommend funding pending a statement by the Applicant's Director (e.g. head of laboratory/department) stating that the results of the funded research will belong to the Investigator, in the form of adequate authorship in any ensuing publications (last name/corresponding author).

Please note that:

- current Telethon grant holders may submit a Telethon-UILDM Clinical Project
- the submission of a Telethon-UILDM Clinical Project does not preclude the possibility of participating in another Telethon call
- a researcher may be funded in a maximum of 4 Telethon-UILDM Clinical Projects (either as the PI/Coordinator for 1 project and as a Partner in 3 Multicentre studies or as a Partner in 4 Multicentre studies). If this limit is exceeded, his/her participation in newly funded studies should be discussed and reconsidered with the Telethon Scientific and Administrative Offices, which will address this issue on a case by case basis.

New Application or renewal by a Former Grantee

A previous holder of a Telethon-UILDM project (either as PI or Coordinator) may submit a new project (or the renewal of a project completed or in its last year of activity) when all of the following apply:

- the Scientific Report of the previous project has been submitted or will be included in the Application
- the Final Administrative report has already been sent or at least 30% of the total funds available for the final year of the previous project has been spent. Total funds include the planned budget plus residual budget from the previous year, if any.

Revised Application

When applying with a revised project (which was not awarded funding in a previous call) a cover letter must be attached highlighting modifications and including replies to the reviewers' comments, if any.

Methodological support

Fondazione Telethon offers the Applicant 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**", by filling out the form provided within the online Application.

The requests received may be evaluated also by the UILDM Medical Scientific Committee members, if indicated by the Applicant, who 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 types of 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 requested and submitted via email to the Telethon Scientific Office, no later than April 27,

2015. 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 Applicant by May 25, 2015. Please note that any support received does not guarantee the success of the Application.

Clinical Research Project

The research project must contain all the scientific and administrative information requested in the guidelines.

Budget

The budget description must be accurate and every item must be justified. Applicants have to fill out only those items that apply to the project.

For clinical projects that entail the enrolment of a number of patients determined on the basis of 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/Coordinator may contact the Telethon Scientific Office for assistance (soffice@telethon.it), if deemed necessary.

Fondazione Telethon wishes to remind Applicants that funds are raised through many small individual donations and recognizes its special responsibility to oversee a precise allocation of funds.

Application Details and Forms

Application forms can be found on the Telethon website (<http://proposals.telethon.it>) from **March 20, 2015**.

The **Pre-Submission Inquiry**, if of interest for the Applicant, has to be filled out in Italian and submitted via email to the Telethon Scientific Office, no later than April 27, 2015.

Applications must be prepared according to the specific guidelines ([Guidelines-Telethon-UILDM-Clinical-Projects-2015](#)), which are an integral part of this call.

- It is mandatory that the Application be approved by the Applicant(s)' Host Institution(s)
- Any external appointments at a foreign Institution must be clearly indicated in the Host Institution section and in the related "Host Institution Agreement" document (see "Required Documents" below)
- All studies including biological material of human origin and/or human subjects must comply with the relevant Italian laws (see "Required Documents" below).

The Application forms must be:

- written in English (this applies to all online forms and uploaded documents except for the lay summary, which must be both in Italian and in English)
- submitted **by July 15, 2015 at twelve o'clock (midday)**
- completed by sending the Administrative Report (for Former Telethon-UILDM PIs/Coordinators of previous Telethon-UILDM projects only) to the email address reportamministrativi@telethon.it by **July 15, 2015**.

The Coordinator of a multicentre project is responsible for both the accuracy and the completeness of all the information and documentation submitted to Fondazione Telethon (including that of the Partners' Centres).

Required documents

Besides completing the Application forms online, **mandatory documents** are to be provided according to the Guidelines.

THE EVALUATION

Applications that are incomplete or do not comply with the rules stated in the present Call for Application and pertinent guidelines will not be processed for evaluation.

Procedure

All complete Applications will undergo a preliminary evaluation in order to verify the relevance of the project to the research goals defined in the Call for Applications.

The projects identified as suitable will undergo peer review by the Telethon-UILDM Clinical Trial Evaluation Committee, an international panel of experts. The complete review process will include independent specialist opinions from appropriate international referees.

The awarding of grants will be assigned on a competitive basis according to the scientific value of the proposal and its clinical relevance. Muscular dystrophies and spinal muscular atrophy will be considered of highest priority.

The Clinical Trial Evaluation Committee will also review the budget, which may be changed with respect to the original request.

Timetable

The awarding of funds will be formally discussed during the plenary Scientific Review Meeting to be held by December 2015.

The PI/Coordinator/Partner will be notified of the evaluation's result by email first and will then be allowed to access his/her Telethon Review Summary online by December 22, 2015.

TELETHON POLICIES AND RULES

Fondazione Telethon, as a member of the International Research Disease Consortium (IRDiRC), endorses and applies IRDiRC's Policies and Guidelines: http://www.irdirc.org/?page_id=12.

In addition, all Telethon Applicants and Grantees are bound to comply with the policies reported below.

Safeguard of research integrity

Fondazione Telethon is fully committed to maintaining public trust in its research enterprise. Therefore, Applicants, Grantees and all research personnel (investigators, trainees, administrators and staff) related to any Telethon Application or Telethon project must strictly adhere to the highest standards of research integrity.

Research integrity includes:

- the use of honest and verifiable methods in proposing, performing, and reporting research results according to rules and regulations that are in force within the international scientific community
- the commonly accepted professional codes or norms.

Identification of research misconduct will result in Fondazione Telethon issuing a formal complaint to the PI's/Coordinator's/Partner's Host Institution, which should then respond according to its own policy for handling allegations of research misconduct.

Awarding of research funds

Awarded funds must contribute towards the costs of the research for which the funds were awarded. Fondazione Telethon requires that the funds be used effectively and economically, and that the expenses be essential for the research for which the funds were awarded.

No funds will be awarded by Fondazione Telethon to any Host Institution, until both parties have signed a specific agreement according to art. 3 of the D.P.R. 20th March 2003, n. 135.

Fondazione Telethon awards funds to the Host Institution's administrative body (External management). Alternatively, Fondazione Telethon can manage the funds (Direct management). This is a free service offered by Fondazione Telethon. In both cases the expenses must be sustained according to the grantee's indications. The Host Institution entrusted with the funds is strongly invited, in the choice between the two forms of fund management listed above, to strictly adhere to the indication expressed by the grantee.

Fondazione Telethon reserves the right to ask for a copy of the Statute of the Host Institution and of the latest available Balance Sheet as well as the acknowledgement of the legal status for all the Investigators working for non-profit private organisations; equally, it can request updates of the Statute and of the Balance Sheet in the case of Investigators working for non-profit organisations that have already benefited from Telethon funds in the past. Fondazione Telethon reserves the right to evaluate case by case the compatibility of the statutory guidelines of the private institutions with the aim of ensuring social benefit and of the financial reliability in order, if necessary, to dictate the conditions of the assignment/payment of the contribution. Fondazione Telethon may decide to impose the Direct Management.

Fondazione Telethon reserves the right to site visit the Host Institution before releasing any funds and at any time afterwards during the funded time period.

The release of funds is subject to the submission of all the necessary documentation as specified in the present Call for Applications and as requested in the administrative letter.

In case of two- or three-year awards, recipients will be expected to provide a detailed yearly administrative report to obtain subsequent yearly payments.

Twenty percent of the final year's budget will be reimbursed on completion of the project, upon submission of the final scientific report and approval of the administrative report, demonstrating that all granted funds have been spent.

Fondazione Telethon reserves the right to cancel payments whenever a conflict arises regarding the stipulations contained in this Call for Applications, or stipulations specifically made by Fondazione Telethon to the Investigator.

Publications, data sharing and final scientific reports

All Grantees are required to:

- acknowledge Fondazione Telethon in all publications arising from the project by specifying the **project number** in the publication and send the relative PDF file by email to papers@telethon.it
- adhere to the Telethon **Open Access Policy** (<http://www.telethon.it/sites/default/files/policy-en-oa-july-13.pdf>)
- deposit data generated from research projects, including source data, in appropriate open or controlled access public databases
- submit a **Final Scientific Report** at the end of the project.

Development of Telethon-funded research results

As Fondazione Telethon's goal is to translate research results into therapies and to make them available to patients, Fondazione Telethon recognises that this can be achieved through the activation of industrial collaborations and partnerships that may accomplish the development, manufacture and distribution of therapies, drugs, diagnostics and devices.

While acknowledging **full ownership of the intellectual property** rights arising from Telethon-funded research to its Grantees and/or Host Institutions, Fondazione Telethon requests that they **undertake any necessary steps to ensure adequate valorisation** and exploitation of such research results.

Furthermore, all Telethon Grantees are required to ensure that the results of the Telethon-funded research projects are **duly protected by a patent application** through direct collaboration with their Host Institutions' Technology Transfer Office, as this may **facilitate the full realization of the translational potential of such results**.

Telethon Grantees and/or their Host Institutions must promptly inform Fondazione Telethon via written communication of any new patent filings related to the Telethon-funded research and of the related prosecution.

Fondazione Telethon wishes to play a proactive role in this process and offers advice and assistance to Grantees and their Institutions through the Telethon Business Development Office, to which any request for further information regarding intellectual property issues and valorisation activities must be addressed (tto@telethon.it).

Privacy of personal data

With the introduction of law 196/2003 regulating the protection of persons and other subjects with regard to the treatment of personal data, Fondazione Telethon, having their head office in Via Varese 16b, Rome, as holder of the information, are legally bound to obtain the consent of the PI/Coordinator/Partner regarding the use of the personal data included in the Application Form. See [Appendix 1](#) for further clarification.

March 20, 2015

FONDAZIONE TELETHON

THE ATTACHED APPENDIX HAS BEEN LEFT IN ITS ORIGINAL ITALIAN AS ITS USE IN THIS CASE APPLIES ONLY TO ITALY

Appendix 1

**INFORMATIVA AI SENSI DELL'ARTICOLO 13 DEL D.LGS. 196/2003
("CODICE PRIVACY")**

La Fondazione Telethon con sede in Via Varese 16b, 00185 – Roma, Titolare del trattamento (di seguito il "Titolare"), La informa che tratterà i dati da Lei forniti al fine di ufficializzare la Sua partecipazione al bando di ricerca Telethon, oltre che di consentire la valutazione del progetto di ricerca ivi presentato e, qualora finanziato, per la gestione amministrativa dello stesso.

Il Titolare la informa altresì della possibilità che, nell'ambito della comunicazione scientifica di Telethon avente ad oggetto il progetto di ricerca da Lei presentato, i Suoi dati, potranno essere diffusi attraverso: i) canali istituzionali: il sito internet www.telethon.it, le newsletter on-line, il Telethon notizie ed ogni altro evento di comunicazione; ii) divulgazione scientifica: libro degli abstract, esposizione poster ecc; iii) direct marketing; iv) mezzi di comunicazione di massa.

Il Titolare tratterà i dati da Lei forniti in ottemperanza degli obblighi normativi, garantendo il pieno rispetto delle norme di legge, contrattuali e dei regolamenti. I dati personali saranno inseriti in un database di proprietà della Fondazione Telethon e verranno trattati mediante strumenti manuali, informatici e telematici, con logiche e modalità volte a garantire la sicurezza e la riservatezza dei dati stessi. I dati trattati potranno essere comunicati a soggetti incaricati, interni o esterni alla Fondazione Telethon e/o ad altri soggetti terzi che saranno nominati Responsabili del trattamento dei dati nei limiti e nel rispetto delle finalità sopra specificate nonché nel rispetto di idonee procedure di sicurezza oltre che diffusi in coerenza a quanto sopra specificato. Il conferimento dei dati è facoltativo ma il rifiuto può comportare l'impossibilità delle finalità predette. In ogni momento Lei potrà rivolgersi al Titolare del trattamento nella persona del Direttore Scientifico presso la Fondazione Telethon, piazza Cavour 1 - 20121 Milano al fine di richiedere la cancellazione, la distruzione, l'aggiornamento, l'opposizione per motivi legittimi, la rettifica, l'accesso o per esercitare gli altri diritti di cui all'art. 7 del Codice Privacy, oppure per richiedere l'elenco aggiornato dei soggetti nominati Responsabili del trattamento.