TELETHON RESEARCH PROJECTS

CALL FOR APPLICATIONS - 2015

General

Fondazione Telethon funds a limited number of research projects (either single- or multicentre) in basic or clinical research aimed at finding therapies for genetic diseases.

Awards are contingent upon the availability of funds.

Eligibility criteria

Eligible diseases

This call is in support of research projects focusing on diseases of proven genetic origin, either monogenic or polygenic forms.

Non-Eligible diseases

- Cancer
- Multiple sclerosis
- Acquired immunodeficiency
- Amyotrophic Lateral Sclerosis (ALS). [Note: funding opportunities for ALS are available through AriSLA (http://www.arisla.org/), a dedicated Foundation supported by Telethon]
- Multifactorial diseases; studies focused on the identification of genetic risk factors (e.g. SNPs or other predisposing variants) for multifactorial diseases are not eligible.

In case of doubt, please contact the Telethon Scientific Office (soffice@telethon.it).

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 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 multicenter project) must prove to be:

- scientifically independent
- resident in Italy.
If the Applicant does not hold a permanent position, the Host Institution is required to declare that it will provide a salary for him/her for the duration of the entire project.

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.

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).

**Telethon Grantees may hold a maximum of one Telethon Research Project grant as PI/Coordinator plus two further grants as Partner.**

The following scheme applies:

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<tr>
<th>Holders of Current Telethon Research Project Grants as:</th>
<th>May Apply as:</th>
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<td><strong>PI/Coordinator</strong></td>
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**Progression of a Telethon Exploratory Project or Program Project**

A Research Proposal that is the progression of a Telethon Exploratory Project can be presented by a PI even if he/she is applying within the present Call with another proposal, or is holding another Telethon Research Project, on a different topic. This possibility is given for two years after the year of the Exploratory Project award. This year it applies to Exploratory Projects awarded in 2012 and 2013.

The possibility to hold two projects on a different topic as PI/Coordinator applies also to the Coordinators submitting an Application for a multicentre Research Project that is the progression of their Telethon Program Project 2010.

**New Application or Renewal Application by a Former Grantee**

The holder of a previous Telethon Research Project may submit a New Application, or the Renewal of the previous project, when all of the following apply:

- the Scientific Report of the previous project has already been submitted or is included in the Application
• at least 30% of the last year’s funds of the previous project has been spent.

The holder of a previous Telethon Exploratory Project may submit a New Application, when all of the following apply:
• the Scientific Report of the Exploratory project has already been submitted or is included in the Application
• at least 80% of the funds from the previous project has been spent.

Revised Application

When submitting a revised Application, the PI/Coordinator must complete the Cover Letter Form.

If the previous Application was excluded by triage, the Cover Letter must highlight the relevant modifications of the revised Application. If the previous Application underwent full review, the Cover Letter must also include a detailed reply to the critiques.

In case the revised Application is submitted by a PI/Coordinator different from the previous one, an explanation must be provided in the Cover Letter.

It is not uncommon for a project to be approved for funding at the second/third attempt. Fondazione Telethon believes, however, that resubmission of a revised Application beyond the third attempt is not appropriate. Therefore, starting with the present Call, only two resubmissions of a previously unsuccessful Application, not necessarily in consecutive years, will be accepted.

For this year only, all revised applications will be accepted, irrespective of how many times they were resubmitted in the past, unless differently communicated to the Applicant as a feedback to a previous Application.

Research Project

The research project must contain all the scientific and administrative information required in the Guidelines, which must be followed carefully.

Budget

The budget description must be accurate and every item must be justified.

Telethon wishes to remind Applicants and their Institutions that funds are raised through many small individual donations. Telethon therefore recognises its own special responsibility to oversee an appropriate allocation of funds.

Application Details and Forms

Application forms can be found on the Telethon website (http://proposals.telethon.it) from November 20th, 2014.

Applications must be prepared according to the specific guidelines (Guidelines Grant Proposals 2015.pdf), 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)
• All studies employing vertebrate animals must comply with the relevant Italian laws and adhere to Telethon’s policy (reported in the Guidelines).

The Application forms must be:
• written in English (this applies to all online forms and uploaded documents)
• submitted by January 15th, 2015 at twelve o’clock (midday)
• completed by sending the Administrative Report, if applicable.

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, lack any of the mandatory documents or do not comply with the requirements stated in this Call for Applications and pertinent Guidelines will not be accepted.

Procedure
All Telethon staff and reviewers involved in the evaluation of Applications are bound by strict confidentiality rules defined by Telethon.

All accepted Applications will be reviewed by the Telethon Scientific Committee (http://www.telethon.it/sites/researchers/scientificcommittee/), an international panel of experts.

Grants will be awarded on a competitive basis.

Reviewers are asked to separately evaluate and score the following two aspects of each Application:

1. Scientific merit (is the proposed research excellent?) - the following parameters will be evaluated:
   • Significance
   • Originality of science
   • Appropriateness of design and methods
   • Preliminary results
   • Feasibility/safety
   • Link to the disease

2. Impact on patients (how close to therapeutic development or to any other potential impact on patients are the proposed studies?)

The two scores will contribute to the final score of the project with a weight of 90 and 10 percent, respectively. Telethon’s Guidelines for reviewers are available online - External Reviewer Guidelines.pdf and Internal Reviewer Guidelines.pdf.

For multicentre studies, special emphasis will be dedicated to evaluating whether each Partner’s contribution is necessary for the proposed project, and whether complementarity and synergy are a strength of the proposal.

A triage step may be adopted if a very high number of Applications is submitted. In the triage step the Scientific Committee will preliminarily rank all the Applications, without exception, on the basis of their scientific competitiveness. Applications that have received low scores will be triaged.

All other projects will undergo complete review and will be evaluated by the Scientific Committee and by appropriate international external referees. Revised Applications will be assigned to the reviewers who
evaluated the previous version, if deemed appropriate, and depending on their availability. The Scientific Committee will also review the budget, which may be reduced with respect to the original request. In particular, care will be taken to avoid overlap funding of scientific activities already supported by other Applicant’s grants, which must be accurately described in the appropriate section of the online Application.

All information regarding the Peer Review Process can be found on our website (http://www.telethon.it/en/about-us/scientific-area/funding/peer-review-process).

Timetable
The awarding of funds will be discussed during the plenary Scientific Review Meeting to be held on June 18th-19th 2015.

Applicants will be notified of the evaluation results by email by the end of June 2015. PIs/Coordinators will be allowed to access their Telethon Review Summary online from July 31, 2015.

The Administrative office will send specific communication to all Grantees by August 7, 2015, describing all administrative steps required to activate the grant.

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. 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 according to the Grantee’s indications (Direct management). This is a free service offered by Fondazione Telethon.
The Host Institution entrusted with the funds is strongly invited to strictly adhere, in the choice between the two forms of fund management listed above, 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 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
- 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

Fondazione Telethon has the goal to translate research results into therapies available to patients and recognizes 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 Fondazione Telethon-funded research to its Grantees and/or Host Institutions, Fondazione Telethon requests that they undertake all the steps required to ensure adequate promotion and seek opportunities of development of such research results.

Furthermore, all Telethon Grantees are required to ensure that the patentable results of Fondazione 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.
Grantees and/or their Host Institutions must promptly inform Fondazione Telethon via written communication of any new patent filings and execution of agreements with for-profit entities related to Fondazione Telethon funded research.

Upon request Fondazione Telethon offers to Grantees and their Host Institutions advice and assistance regarding intellectual property issues and technology transfer activities through the Telethon Business Development Office (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 dei Magazzini Generali 18/20, 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.

**November 20th, 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 dei Magazzini Generali 18/20, 00154 – Roma, in fase di trasferimento a decorrere dal 1° gennaio 2015 in Via Varese 16, 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 di 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 a 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 dell’Ufficio 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.