

FONDAZIONE



TELETHON RESEARCH PROJECTS

CALL FOR APPLICATIONS - 2017

General

Fondazione Telethon funds a limited number of research projects in basic and clinical research ultimately aimed at finding therapies for genetic diseases.

Only applications for single-center proposals are admitted.

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, of** 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 also by Fondazione Telethon
- Multifactorial diseases (i.e. studies focused on the identification of genetic risk factors, e.g. SNPs or other predisposing variants).

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.

Eligible Applicants

In order to be eligible for funding, the Principal Investigator (PI) must prove to be scientifically independent.

If the Applicant does not hold a permanent position, the Host Institution should be aware that the salary of the Investigator cannot be requested in the Budget of the application.

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).

A dual appointment in a foreign is allowed only if it does not impinge on the effective conduct of the Telethon research project.

New Application or Renewal Application by a former grantee

The holder of a previous Telethon research project may submit a New or a Renewal Application, only if the previous grant is completed or is in its last administrative year. Partners of currently funded projects can apply regardless of the year of the grant.

Revised Application

When submitting a Revised Application, the PI 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 one. 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 different PI, an explanation must be provided in the Cover Letter.

Only up to two resubmissions of a previously unsuccessful application, not necessarily in consecutive years, **will be accepted**.

Project duration

The duration of the proposed project can range from 1 to 3 years.

Budget

A maximum of 80,000 €/year is allowed. The budget description must be accurate and every item must be justified; requests above 60,000 €/year will be scrutinized with particular attention.

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

Application details and forms

Application forms are available on the Telethon grants portal (<http://proposals.telethon.it>) from **November 15th, 2016**.

The Full Application comprises the **Core Project** and the **Supplementary Contents**.

Applications must be prepared according to the specific guidelines ([Guidelines Grant Proposals 2017.pdf](#)), which are an integral part of this Call.

Specific Requirements:

- It is mandatory that the application be approved by the Applicant's Host Institution
- Any external appointment at a foreign Institution must be clearly indicated in the Host Institution section and in the related "Host Institution Agreement" document (see "Required Documents" in the Guidelines)
- All studies using biological material of human origin and/or involving human subjects must comply with the relevant Italian laws (see "Required Documents" in the Guidelines)
- 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 18th, 2017 at 1:00 p.m.

Access to the Cineca High Performance Computing infrastructure and services

On the basis of an existing agreement between Fondazione Telethon and Cineca, Telethon investigators may benefit from Cineca's High Performance Computing (HPC) infrastructure, data management services and analysis tools for applications such as Next Generation Sequencing (NGS) data analysis and molecular dynamics simulations. Available HPC resources and services are listed on the Cineca website: <http://www.hpc.cineca.it/content/hpc-science>.

Applicants requesting access to Cineca's HPC tools and services are asked to fill in the dedicated form. Applications that are approved for funding by the Telethon Scientific Committee will be forwarded to Cineca for the validation of the request. The feedback from Cineca will be included in the Telethon Review Report.

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 and conflict of interest rules defined by Telethon.

All accepted applications will be reviewed by the Telethon Scientific Committee (<http://www.telethon.it/en/what-we-do/our-method/scientific-medical-committee>), an international panel of experts.

Revised Applications will be assigned to the reviewers who evaluated the previous version, if deemed appropriate, and depending on their availability.

Grants will be awarded on a competitive basis.

The Peer Review process entails two consecutive phases: Triage and Full Review.

Triage

Only the **Core Project** section, consisting of **General Information, Overview, Scientific Approach** and **Cited Literature**, will be made available to the Telethon Scientific Committee members for evaluation at this stage. All applications, without exception, will be ranked based on scientific competitiveness and up to 70 of the top ranking projects will proceed to the second review phase.

Full review

The top ranking projects will undergo complete review; in this phase, the **Full Application** (Core Project plus Supplementary Contents) will be evaluated by selected Scientific Committee members and by appropriate international external referees.

Reviewers are asked to separately evaluate and score the proposals on the following two aspects:

A) Scientific merit (is the proposed research excellent?) - the following parameters will be evaluated:

- Link to the disease
- Significance
- Innovation
- Appropriateness of design and methods
- Preliminary results
- Feasibility
- Safety
- Experience of the Applicant in the field.

B) 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](#).

The top scoring proposals will be discussed by the Scientific Committee during the Plenary Review Meeting and all Committee members will provide a final score based on the two aforementioned parameters.

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 overlapping funding of scientific activities already supported by other Applicant's grants, which must be accurately described in the appropriate section of the online application.

Timetable

Applicants whose application is triaged will be notified by May 31st, 2017.

The awarding of funds will be discussed during the plenary Scientific Review Meeting to be held on June 22nd- 23rd, 2017.

Applicants whose application undergoes full review will be notified of its outcome by email by the end of June, 2017, and will be allowed to access the Telethon Review Report online starting from July 31st, 2017. At the same time, the Telethon Administration Office will communicate to all successful Applicants the administrative details and instructions to activate the approved grants. Successful Applicants will also be required to promptly provide a lay summary in both Italian and English.

TELETHON POLICIES AND RULES

Fondazione Telethon, as a member of the International Research Disease Consortium (**IRDiRC**), endorses and applies IRDiRC's Policies and Guidelines, which "emphasize the collaboration in rare diseases research, the involvement of patients and their representatives in all relevant aspects of research, as well as the sharing of data and resources" (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 projects and results according to rules and regulations that are in force within the international scientific community
- the commonly accepted professional codes or norms.

Potential research misconduct will result in Fondazione Telethon issuing a formal notification to the PI'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 be spent to accomplish the research for which they have been approved. 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 will perform a Direct Management of funds at no additional costs. Exceptionally, and for valid and justified reasons, the Host Institution can ask Fondazione Telethon the possibility to manage itself exclusively the funds for personnel and overheads (External Management).

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 the compatibility of the statutory guidelines of the private institutions on a case by case basis, with the aim of ensuring social benefit and of the financial reliability in order, if necessary, to dictate the conditions for 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 grant period.

The release of funds is subject to the submission of all required documentation, as specified in the present Call for Applications and as requested by the Administrative Office.

In case of two- or three-year awards, recipients must provide a detailed yearly administrative report in order to obtain subsequent yearly payments.

Fifty percent of the final year's budget will be reimbursed upon completion of the project, 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 Acknowledgements/Funder section and send the relative PDF file to papers@telethon.it
- be compliant with the Telethon **Open Access Policy** (<http://www.telethon.it/en/research/for-researchers/open-access>)
- 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, manufacturing and distribution of therapies, drugs, diagnostics and devices.

While acknowledging that full ownership of the intellectual property rights arising from Telethon-funded research belongs 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.**

Accordingly, all Telethon Grantees are required to ensure that the patentable results of Telethon-funded research projects are duly protected by a patent application through the interaction and cooperation with their Host Institutions' Technology Transfer Office before results' disclosure, 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 Telethon's funded research.

On a case by case basis and at terms and conditions to be mutually agreed between Fondazione Telethon and the Host Institution, Fondazione Telethon reserves the right to obtain an exclusive mandate to promote and negotiate the exploitation of the results of Telethon-funded research projects with third parties.

Furthermore, Fondazione Telethon offers advice and assistance regarding intellectual property issues and technology transfer activities through the Telethon Business Development Office to those Grantees and Host Institutions that cannot take advantage of a Technology Transfer Office. For further information, please contact the Telethon Business Development Office at: 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 its head office in Via Varese 16b, Rome, as holder of the information, is legally bound to obtain the consent of the PI regarding the use of the personal data included in the application form.

See Appendix 1 for further clarification.

November 15th, 2016

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 16/b - 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 che, qualora il progetto di ricerca da Lei presentato sia finanziato, i Suoi dati potranno essere diffusi per finalità di comunicazione di Telethon attraverso: i) i canali istituzionali (es. il sito internet www.telethon.it, le newsletter on-line, la rivista Telethon notizie ed ogni altro evento di comunicazione); ii) la divulgazione realizzata in occasione di eventi scientifici (es. la Convention Telethon), ovvero attraverso canali anche web di soggetti terzi quali, per esempio, enti non profit che hanno tra le loro finalità quella di informare i pazienti sulla ricerca in corso sulle loro malattie; 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 Scientifico presso la Fondazione Telethon, Via Carlo Poerio, 14 - 20129 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.