FONDAZIONE



Multi-round Telethon Call for Research projects 2021 – 2024

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GUIDELINES FOR PREPARING AND SUBMITTING THE ONLINE APPLICATION

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 completing the Application). 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 or word number limitations specified.

Maximum number of characters in the different sections includes spaces.

Registration

To register, fill in and submit the Application, refer to the *TETRA Portal Instructions.pdf* - <u>System Help</u> available on the Home page.

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.

New Application

On the Home page, clicking **My Application** on the left menu, then clicking on the New Application box on the right, Applicants can access the page listing all the available Calls for Applications. Click *Apply* on the "Multi-round 21-24 – Round 1 2022 **Track Basic**" or "Multi-round 21-24 – Round 1 2022 **Track PoC**" link to create a new Application form. Please visit our site for further information on the two different tracks.

<u>Please note</u> that by choosing the desired *track* you are directly applying to the relative dedicated peer review process and competition. If the project does not fit the selected track, it will not be considered eligible for the peer review.



Completing the Application

The created Application is listed in *My Applications* (link on the left-hand menu of the Home page).

In the *My Applications* home page, at the right, the following button sections are shown:

Edit

Click on the 'Edit' button if you wish to make any changes to your Application.

View/Print

Click on the 'View/Print' button to generate the Application form as a PDF file. A PDF of the Application can be downloaded at any time by clicking on this button.

Validate

To validate the Application click 'Validate'. In the "Validation" section within the Application form eventual errors will be listed.

Submit

The Application form cannot be submitted until it has been validated to ensure that all required fields have been entered, and the data meet the submission requirements. The 'Submit' button is active once the Application has been correctly validated.

Delete

If you are sure you wish to delete the Application form, click on the 'Delete' button. Please note: once an Application has been deleted it cannot be recovered.

To navigate into the Application sections, at the top of the Application page, the following buttons are shown:

Cancel, Previous, Next, Save, Save and Close.

Clicking the Cancel button all modifications are not saved and you are returned to My Application home page. **Previous** and **Next** buttons allow you to navigate into the Application, the **Save** button saves all modifications done, remaining on the Application page you are working on, while clicking on Save and Close you can save, exit the Application page and return to the Application form afterwards as often as you like.

Mandatory fields are indicated by red dots. To successfully submit an Application, all mandatory fields must be completed. Any item missing before submission is listed in the *Validation* section.

When the Application is validated, the Lead Applicant may *Submit* the Application, which will have to be approved by the Organisation Approver for the final submission (see detailed guidelines below, Organisation Approver Invitation). Before the Organisation Approval, the Application is displayed in My Applications home page in the status: Awaiting Signatory Approval. The Application status will change in *Submitted* only after the approval by the Organisation Approver.

Upon Application Approval and Submission by the Organization Approver, a final Application number will be assigned to it. Please refer to this number in any future communications related to it.

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.

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The Application comprises:

- o General information
- o Overview
- Overall Description of the Research Project (For Triage Phase evaluation)
- o Research Plan
 - Research Plan (document upload)
 - Human Subject
 - Vertebrate Animals
- Collaborators
- o Personal Data and Curriculum Vitae
 - Biosketch (document upload)
 - ID Research Platform, Personal author ID
- Budget and Personnel
- Other Financial Support
- Host Institution
- Reviewers
- Notes
- Declaration
- Validation

General Information

Project Title – Please insert the title of the proposed project.

Project duration – (12 or 24 months)

Type of Applicant

New Applicant is a researcher who has never applied to a Telethon call;

Former Applicant is a researcher who has already applied to a Telethon Call but has never been funded; Former Grantee is a researcher who has already been funded by Telethon in the past.

Type of Application

Only the "New Application" option is available for this round.

Applicant's details – Automatically retrieved from the applicant's Basic Details (Manage My Details section)

Overview

Abstract (max 250 words) - Provide the following information:

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

Lay Abstract – English (max 250 words)

Summarize the project using an English lay language. This description is very important, it is meant to describe the project to lay people and will eventually serve for lay communication reasons after award and, as such, it may become public information. Therefore, do not include proprietary/confidential information.

Project Title - Italian

Insert the title of the project in Italian.



Lay Abstract – Italian (max 250 words)

Summarize the project using an Italian lay language. This description is very important, it is meant to describe the project to lay people and will eventually serve for lay communication reasons after award and, as such, it may become public information. Therefore, do not include proprietary/confidential information.

Please consider that the lay abstracts are fundamental parts of the application and Fondazione Telethon may slightly modify both Lay Abstract texts for communication purposes and that these information will be available on Fondazione Telethon website in case of projects approved for funding.

MeSH terms, Disease Name, Orpha Number, Orphanet classification, Disease OMIM number, ICD-11 code.

Area of Research – Please check all that apply **Research Type** – Please check all that apply

Overall Description of the Research Project

(Please note that the triage review phase will focus on this section)

Please describe central hypothesis, objectives, specific aims, research design, methods, and potential outcomes (max 8,000 characters)

Please describe the central hypothesis that the proposed research seeks to address. Please include overall objectives and experimental approaches of the proposed research. Please identify the potential outcomes of the proposed research.

Research Plan

Research Plan upload - Please refer to the "Research plan" template (Annex A - RESEARCH PLAN of this document), compile it, and upload it as a .pdf in the Research plan upload section. – A sample template will be also available to download in the header of the online application form.

- Please use Times New Roman, 11 Fill in all the sections
- This document should <u>not exceed 12 pages</u> (figures included, GANTT, Relevant Publications and Cited Literature **excluded**).
- Please keep the .pdf size below 25 MB, to avoid overloading our servers. Use high-resolution pictures only for photographs that require details.



Introduction



Indicate the starting and ending stage of development of the proposed project, referring to the above **Telethon Development Pathway (TDP)** – Please note:

- If you apply for the Track Basic Research → only steps 1 to 3 are applicable (as starting stage)
- If you apply for the Track Preclinical PoC → only steps 4 to 5 are applicable
- **A.** Hypothesis, Background and Rationale Clearly state the main hypothesis to be tested and the rationale of the proposed research. Critically evaluate the existing knowledge and identify the specific gaps in the relevant field.
- **B.** Preliminary Results 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 a project. Results are considered 'preliminary' only if unpublished.
- C. Research Plan, Research Design and Methods List the specific aims of the project.

For each aim provide the following information:

- Title
- Brief Description What is the question being asked? What is the general experimental design?
- **Experimental Plan** How are you going to address this aim? Please provide an extensive description of the experimental approaches.
 - Explain the need for **collaborations** (if any) to achieve the scientific aim. Indicate how the idea of collaborating originated, the different approaches 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.
- Expected Outcomes Describe the expected outcomes.
- **D.** Feasibility, possible Pitfalls and alternative Approaches Please explain how the proposal is focused on achieving specific and feasible goals. In addition, please state which pitfalls could arise during the research activity and the alternative plans that will be implemented.
- **E. Significance and Innovation** Describe how the scientific knowledge will be advanced if the aims of the project are achieved. Indicate if the project employs novel concepts, approaches or methods and if it challenges existing paradigms in the field or develops new methodologies or technologies.
- **F. Possible evolution of research, if successful** Please provide details for future development of the proposed research.



- G. Unmet need and relevance to Fondazione Telethon's mission Please explain how the results of this research fill a gap in knowledge or meet an unmet need. Clearly specify how the goals of the project fit with Fondazione Telethon's mission (http://www.telethon.it/en/what-we-do/our-mission).
- H. Background Intellectual Property Please indicate any intellectual property where the Applicant is an inventor and any intellectual property owned by the Applicant or by a third party that would be necessary for the development of an approach to treat the disease/syndrome based on the results of the proposed project. For Applicant's intellectual property, please indicate freedom to operate for such development.
- I. Financial Interests Disclosure Please indicate 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.
- L. GANTT Chart Please include a GANTT chart describing the timeframe foreseen for the different specific aims and their components.

M. Cited Literature

List all references. The list must include the name of all authors, year of publication, title, book or journal, volume number and page numbers.

Concise references are not allowed.

The complete list of references will be visible to Reviewers.

Human subjects – Indicate whether the study involves:

- 1. Human samples from a collaborator site or an external biobank
- 2. Human samples from individuals referred to the PI's Host Institution
- 3. No human samples or subjects

Vertebrate animals - Specify whether activities involving vertebrate animals are planned at any time during the proposed project.

→ If yes please indicate whether the Ethical Authorization documentation is already in place, in progress or not present.

Should the grant be approved for funding, funds will not be provided until the pertinent Ethical documentation has been obtained. Please activate in due time all necessary procedures to obtain this approval in accordance with the relevant Italian laws.

Be aware that the relevant approval documents must be provided for grant activation.

Telethon rules and policy on animal experimentation

Telethon recognizes that experiments on animals are often necessary in many areas of biomedical research. Proposals submitted for the evaluation MUST explain why the scientific objectives cannot be achieved without using animals.

Where experiments using animals are necessary, you are required to strictly adhere to the relevant Italian laws, rules and regulations (D.to L.vo 26/2014); moreover, approval by your Institution Ethics Review Body is mandatory. The ethical review process is a means of ensuring that any use of animals within lab animal facilities is carefully considered, adequately justified and carried out as humanely as

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possible, so that any adverse effects experienced by the animals are more than offset by the benefits that arise from the study.

Measures should be put in place to avoid unnecessary duplication of research/testing and fully implement the **Three Rs** (Reduction, Replacement and Refinement, from The Principles of Humane Experimental Technique, Russell and Burch, 1959), from the moment it is recognized that an animal experiment will take place, through the period where the animals are sourced and arrive at the facility, and up to the time they are either dead or have been re-homed. This includes optimizing standards of animal husbandry and care and effective training, supervision and management of all personnel involved. Microbiological status is important not only because there are welfare imperatives in minimizing the incidence of disease but also to avoid the risk that subclinical infections affect research results.

Provide a detailed description of the proposed use of the animals in the work outlined and identify the species, strains, ages, and sex of animals to be used in the proposed work. Provide information on the veterinary care of the animals involved.

Make sure that the fewest animals compatible with obtaining a valid scientific result are used. In this regard, in planning your experiments you should carefully estimate the number of animals needed. You should take into account the likely magnitude of the effect you will be studying and the frequency with which that effect will be achieved for given levels of statistical significance and power. It is unacceptable to base the number of animals to be used solely on the calculation of the number of experiments that can be carried out at any given time. It is also unacceptable to state that the numbers are based on "previous experience" without additional justification, or to answer the question on numbers of animals to be used by paraphrases such as "these numbers are chosen as the minimum necessary to achieve statistical significance". Too few animals are just as unsatisfactory as too many.

Be aware that the relevant approval docs must be provided for grant activation.

Collaborators

The Applicant should list all the collaborators and the required information (Name, Institution, Department, Laboratory, Contribution to the project, Collaboration letter).

Active 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 to the lead applicant a collaboration letter, written in English, which have to be uploaded in the online Application by the applicant himself/herself (see also TETRA Portal Instructions.pdf - System Help.).

Personal Data and Curriculum Vitae

Please refer to the "Biosketch" template (Annex B – BIOSKETCH of this document), compile it, and upload it as a .pdf in the Biosketch upload section. – A sample template will be also available to download in the header of the online application form.

The biosketch may not exceed 5 pages, including the table at the top of the first page, excluding relevant publications. Figures, tables or graphics are not allowed.



Name – Fill in the name of the Applicant.

Position Title – Fill in the present position title of the Applicant.

Education/Training - Complete the education block. Begin with the baccalaureate or other initial professional education. Include postdoctoral, residency, and clinical fellowship training, as applicable, listing each separately. For each entry provide:

- the name and location of the institution
- the degree received (if applicable)
- the month and year of end date (or expected end date).
- the field of study (for residency entries, the field of study should reflect the area of residency training)

A. Personal Statement – Briefly describe why the applicant is well-suited for his/her role(s) in the project. Relevant factors may include aspects of his/her training; previous experimental work on the specific topic or related topics; technical expertise; collaborators or scientific environment; and/or past performance in the field or in related fields, including ongoing and completed research projects from the past three years that the applicant wants to draw attention to.

The applicant may cite up to four publications or research products that highlight his/her experience and qualifications for the proposed project.

B. Positions, Scientific Appointments and Honors – List in reverse chronological order all current positions and scientific appointments both domestic and foreign, including affiliations with foreign entities or governments. This includes titled academic, professional, or institutional appointments whether or not remuneration is received, and whether full-time, part-time, or voluntary (including adjunct, visiting, or honorary).

List any relevant academic and professional honors.

C. Contributions to Science

Briefly describe up to five of the applicant's most significant contributions to science. The description of each contribution should be no longer than one half page, including citations. These contributions do not have to be related to the project proposed in this application.

For each contribution, the applicant may cite up to four relevant publications or research products. If the applicant is not the author of the product, indicate what his/her role or contribution was.

Note that while manuscripts that have not yet been accepted for publication may be mentioned as part of the contribution, only published papers can be cited to support each contribution.

Moreover, the applicant may indicate the historical background that frames the scientific problem, the central finding(s), the influence of the finding(s) on the progress of science or the application of those finding(s) to health or technology, his/her specific role in the described work.

D. Relevant Publications – Please list up to 10 publications relevant to the application.

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 generate an ORCID ID (http://orcid.org/).



Budget and Personnel

A maximum of 80,000 €/year is allowed for the Track Basic Research.

A maximum of 120,000 €/year is allowed for the Track Preclinical PoC.

Budget can cover salaries up to **50%** of the direct costs of the total budget, **for personnel (including the lead applicant) not holding a permanent position.**

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.

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) 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.**

To **ADD** an item, click on the relative button and fill in the required information.

Direct costs

The following expenses associated with the proposed research are allowed:

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 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.

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.

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.



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

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.

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.

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 (input the value) up to 10% of the direct research costs per year.

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

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

- Full salaries for personnel who already receive a regular wage (i.e., permanent position)
- 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.

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 2,000 characters)
- If applicable, specify possible overlaps with the proposed project (max 1000 characters).



Host Institution

Applicant - Provide all the information required. If the Applicant is not the Chief of the Laboratory, the **Independence statement** must be uploaded (max 1,000 characters).

Please note that the Independence Statement **must be provided and signed by the Head of the Lab** and should contain the name of the Applicant, the Application Title and a clear statement highlighting the Applicant's capability to carry out the proposed research in due time.

It is mandatory that any foreign appointment of the Applicant be clearly indicated in this section.

Facilities and Resources - Provide all the information required and list all the key facilities available for implementing the project.

Organisation Approver Invitation

The Organisation Approver will have to be a person (Institution's Director or Responsible Official or Administrative Representative) who, representing the Organisation where the research project will be performed, will have the responsibility for the Application Approval and final Submission.

Click on *Add Contact* to invite the Organisation Approver, who will be notified by mail. A message for the approver can be added by the applicant while inviting.

If the Organisation Approver is not already available in the menu in the IntelliSence menu, add the new one clicking on **Add Person** and save it. Personal email accounts are not accepted, please make sure that the Organisation Approver's email is the **institutional account**.

Please confirm that you wish to include this Organisational Approver then click on **Send the Invitation**.

In the *Organisation Approver* box the Approver's details and the approval status are shown; in case of error the Approver can be deleted clicking on the **Remove** button.

The selected Organisation Approver will receive an email alerting that she/he has to take on the Application Approver role.

Organisation Approver Confirmation and Guidelines

The invited Organisation Approver will receive an email containing the link to access the TETRA platform. In case of first access to the platform, please follow the registration instruction on the webpage; if already registered, clicking on the link in the invitation email opens a page where the Approver can **Confirm** the participation or **Reject** the participation.

After logging, the Organisation Approver will have access, through the left-hand menu *My Approval*, to the Application(s) for which he/she has been invited to approve. For each Application, clicking on the lens icon on the right, the Application page will be shown.

In the central box the *Signatories* section shows the Application approval status. When the Organisation Approver has accepted the invitation, *Confirmed participation YES* will be displayed.

While the Application is in preparation and not yet submitted by the Applicant, the *Signatory approval status* shows *Approval Not Yet Required*; when the Application will be complete and submitted by the Applicant the status will become *Approval Required*.

On the right-hand menu, the Approver can access the following buttons

View/Print

Please click on the 'View/Print' button to view and read the Application. If you agree with the content of the Application please approve below. The Application will then be submitted to Fondazione Telethon. If you do not agree with the content, please Reject Application to return the application to the applicant for amendment.



Approve

To approve the Application, the Approver will have to accept all the following clauses which will be shown in the Approval page:

- I, Organisation Approver, in the role of/on behalf of the Institution's Director or Responsible Official or Administrative Representative agree with the following statements:
 - The Organisation is eligible: it has to be either a public or a private Italian non-profit research Organisation.
 - The Applicant is authorized to submit the Application on the Organisation's behalf.
 - The Organisation undertakes to respect any and all conditions under the Call for Application and discharges Telethon from any liability related to any breach of said conditions by the Applicant and/or by the Organisation itself.
 - The Organisation declares that Fondazione Telethon will be appointed as data processor by the Organisation for the Direct Management of funds.
 - The Organisation declares to have provided the Applicant and any researcher involved in the Application (Key Personnel or Collaborator) with the information attached to the Call for Application in Appendix 1. It also declares that the relevant consents have been gathered in order to be authorized to indicate the personal data of the Applicant and any other researcher in the Application.
 - The Organisation declares to be aware that Fondazione Telethon can, at any time, request the abovementioned documents (Information to data subjects and declaration of consent) and that the Organisation shall deliver any relevant document, according to Fondazione Telethon's request.
 - The Organisation declares to have read the research Application pre-submitted on-line to Telethon by the Applicant and that it is complete and correct.
 - The Organisation declares that it will provide the necessary facilities and personnel to carry out this research project. If the Applicant is not holder of a permanent position, FT can provide salary coverage for the Lead Applicant and for the personnel involved up to 50% of the direct costs of the total requested budget.
 - If the Applicant has stated (see Host Institution Box Applicant) to have a dual appointment in a foreign Institution, the Organisation acknowledges such statement and it declares that the double appointment does not conflict with the time commitment indicated by the Lead Applicant within the Application for an effective conduct of the proposed research project.

Once approved, the Application will be automatically submitted; in the My Application Applicant Home page and in the *My Approval* Approver Home page, the status shown will be Submitted.

Reject

It is possible to explain the reasons for Rejection, which will be emailed back to the Applicant.

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. Coauthors in scientific publications and/or individuals who have been associated with the Applicant and/or his/her collaborators within the last 5 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.

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Notes (max 5,000 characters)

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 certify that the information included in the Application is accurate and complete.
- The Applicant certifies that he/she is entitled and/or authorized to disclose all information provided within the Application.
- The Applicant declares to have provided any collaborator involved in the Application with the information about Data processing attached to the Call for Application in Appendix 1. He/She also declares that the relevant consents have been gathered in order to be authorized to indicate the personal data of any other collaborator in the Application.

Please duly fill in, sign and upload the Applicant's Information about Data Processing (see Call Document – Appendix 1).

Validation

This section will be automatically compiled, once the *Validate* button in the *My Application* Home Page has been clicked. Please note that the sections highlighted in green are those correctly filled in, while those in red have to be completed and key data are still missing.

Application submission and Organisation Approval

The deadline for online submission is June 30th, 2022 at 1:00 p.m.

Please consider that clicking the *Submit* button, will generate a Pre-Submission Application which cannot be further modified.

The Pre-Submission will then automatically be notified to the Grant Organization Approver who, after having checked and accepted the *Host Institution Agreement Clauses* will approve or reject the proposal. If **approved**, the <u>Application will be automatically submitted by the Organization Approver</u>; if **rejected** (the reasons for rejecting may be written back to the Applicant) the Applicant will receive a notification in order to eventually amend the Application. The updated Pre-Submission will then need to be further approved.

FOR THESE REASONS, PLEASE MAKE SURE TO PRESUBMIT YOUR PROPOSAL IN DUE TIME TO ALLOW THE APPROVAL PROCEDURE TO BE CARRIED OUT.

THE DEADLINE IS INTENDED FOR THE APPROVAL OF THE APPLICATION by the Organisation Approver.

Before the Pre-Submission, download the PDF of your Application to check all the sections; in particular check that all images included in the PDF are clearly legible. Please note that the Applicant is liable for the contents and quality of the Application in its final version.

Fondazione Telethon holds the responsibility and authority in making the final decision on the Application's completeness and eligibility.

March 10th, 2022

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Annex A - RESEARCH PLAN

Intro.

| Basic Research Preclinical PoC | | | | | | | |
|--|--|--|--|--|--|--|--|
| Research on mechanisms/ the disease Target Identification 2 Target Validation 2 Target Validation 1 Target Validation 2 Therapeutic approach identification 1 Therapeutic approach identification 2 Therapeutic approach identification 1 Therapeutic approach identification 2 Therapeutic approach identification 2 Therapeutic approach identification 3 Therapeuti | | | | | | | |
| Indicate the proposed starting and ending stage of development of this project, referring to the above Telethon Development Pathway (TDP) | | | | | | | |
| | | | | | | | |
| A. Hypothesis, Background and Rationale | | | | | | | |
| | | | | | | | |
| B. Preliminary Results | | | | | | | |
| | | | | | | | |
| C. Research Plan, Research Design and Methods | | | | | | | |
| Aim 1. | | | | | | | |
| Title: Brief Description | | | | | | | |
| | | | | | | | |
| Experimental Plan | | | | | | | |
| | | | | | | | |
| Expected Outcomes | | | | | | | |
| | | | | | | | |
| Aim 2. Title: | | | | | | | |
| Brief Description | | | | | | | |
| | | | | | | | |

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| Experimental Plan |
|---|
| |
| Expected Outcomes |
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| |
| D. Feasibility, possible Pitfalls and alternative Approaches |
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| E. Significance and Innovation |
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| F. Possible evolution of research, if successful |
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| G. Unmet need and relevance to Fondazione Telethon's mission |
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| H. Background Intellectual Property |
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| I. Financial Interests Disclosure |
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| L. GANTT Chart - Please attach a GANTT chart describing the timeframe foreseen for the different specific |
| aims and their components. |

M. Cited Literature



| Annex | R _ | RIO | ςĸ | FΤ | \sim L |
|-------|-----|-----|----|----|----------|
| Annex | B - | BIU | 7 | ГΙ | ۱.г |

| NAME: | | | | | | | | |
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| POSITION TITLE: | | | | | | | | |
| EDUC | ATION/TRAINING | | | | | | | |
| | INSTITUTION AND LOCATION | DEGREE (if applicable) | Completion Date MM/YYYY | FIELD OF STUDY | | | | |
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| A. | Personal Statement | | | | | | | |
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| В. | B. Positions, Scientific Appointments, and Honors | | | | | | | |
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| C. | Contributions to Science | | | | | | | |
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| D. | Relevant Publications | | | | | | | |
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