



## MULTIROUND CALL FOR RESEARCH PROJECTS

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### 2025-2027 – ROUND I

### EVALUATION GUIDELINES 2025 – COMMITTEE MEMBERS

The present Call supports research projects focusing on rare diseases of proven genetic origin, of either monogenic or polygenic forms. The Call aims at funding basic and pre-clinical research projects focused on rare genetic diseases and conducted by researchers working in Italian public or private non-profit research institutions.

Research projects can be submitted into one of the following tracks:

- **Track BASIC RESEARCH**  
Focused on the identification of disease mechanism(s) and/or disease target(s).
- **Track PRECLINICAL PROOF OF CONCEPT**  
Focused on the identification and validation of therapeutic candidate(s).

#### 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 Fondazione Telethon
- Multifactorial diseases (i.e., studies focused on the identification of genetic risk factors, e.g., single nucleotide polymorphisms or other predisposing variants).

#### Committee Members' Role

Fondazione Telethon (FT) Scientific Committee members (Committee Members) will support the evaluation process, by providing written comments and an overall recommendation for each eligible Application.

#### General Instructions

The Applications and the Evaluation Form are available on *Fondazione Telethon Grant Management* system portal at this [link](#), accessible through personal login and password.

#### Reviewer Account

*Registered Users in TETRA (the former Grant Management system)*

Internal Reviewers already registered in TETRA – the former Fondazione Telethon Grant Management system – are **kindly asked NOT to create a new account**.

Please click on **Forgot Password?** and follow the given instructions for setting a New Password, then enter the portal.

### *New Reviewer to Fondazione Telethon Calls*

To register and review the Applications, Reviewers should click on the **Register Here** button and enter their email address. To complete the registration process, follow the online instructions.

## **Peer Review Process**

### *Letter of Intent - Triage Phase*

The **Triage Phase** is the first phase of the review process and is applied to select the top-ranking projects for a full evaluation. In the triage phase, each Letter of Intent (LoI) is assigned to three members of the FT Scientific Committee, who are asked to score it based on scientific quality. LoIs are then ranked, and the top-ranking Applications will undergo Full review.

After Triage, for the **Full Review** of the top-ranking projects, three Scientific Committee members will evaluate the Applications with the support of two appropriate international External Reviewers.

### *LoI - Triage Instructions*

Committee Members are requested to access *Fondazione Telethon Grant Management* system portal at this [link](#) through personal login and password. By clicking on **Pending Triage**, the Committee Members will find the list of all the Applications assigned to them and can access each LoI's evaluation form by clicking on the specific project. For each project, the Committee Members will find information about the project in the **General** tab, and the LoI will be visible by clicking on the **View/Print** button next to **Application Preview**.

According to the Triage Scoring Scale reported below, Committee Members will select one of three score ranges, and then will manually assign a numerical score in the **Triage Score** section of the form. The score will reflect the recommendation of whether the project deserves full review. When ranges 1.0 – 3.5 or 3.6 – 4.5 are selected, the Committee Members are asked to select the **Weaknesses** (multiple choices are allowed).

The list of weaknesses consists of the following items:

- not hypothesis-driven
- inappropriate design and methods
- not feasible
- weak preliminary studies
- poor rationale
- weak genetic link
- other (please specify in the comments)

Committee Members are also asked to fill in the **Comments on Weaknesses** section (max 3,000 characters). In the **Comments on Weaknesses** section, we kindly request each Committee Member Reviewer to provide a few sentences to highlight any issues regarding the project. **Please note that Committee Members' comments will be used to provide the Applicants with a written overall justification in case of triage exclusion of their proposal.**

When all parts of the evaluation form have been completed, the Committee Members will click on **Submit** and the Application will be listed in the **Submitted Reviews** tab on the Home Page.

| Triage Scoring Scale |                    |  |
|----------------------|--------------------|--|
| Triage Score         | Project Evaluation | Recommendation   |
| 4.6 – 5.0            | Outstanding        | No concerns, suggest for Full Review                           |
| 3.6 – 4.5            | Good to Excellent  | Some concerns (please justify), could undergo full review      |
| 1.0 – 3.5            | Poor to Average    | Major Problems (please justify), not suggested for full review |

### Full Review

Applications that **pass the Lol - Triage Phase** will undergo **Full Review** and will be evaluated by three Committee Members.

In support of their evaluation, Committee Members will be provided with written comments by two appropriate External Reviewers, who will be chosen *ad hoc* for each Application by FT Scientific Officers.

During this phase of the Review process, the *Primary Reviewer* is responsible for the project's presentation during the plenary review session. *Primary* and *Secondary Reviewers* have to provide written comments, whereas the *Tertiary Reviewer* is not requested to (but may provide written comments, if he/she wants to).

Written comments are an essential part of the review and are critical in developing summary statements for the Applicants.

The individual written comments will be anonymously incorporated into a complete review report that will be returned to the Applicant. It is therefore important that the written material is accurate, clearly written, and does not include derogatory language.

Please note: External Reviewers' written comments will also be included as such in the Review Report.

### Full Review Instructions

Three Committee members will review the assigned Applications, and each reviewer is asked to provide a score.

Committee members are requested to access *Fondazione Telethon Grant Management* system portal at this [link](#) through personal login and password. By clicking on **Pending Panel Review**, the Internal Reviewers will find the list of all the Applications assigned to be reviewed and can access each proposal's evaluation form by clicking on the specific project. The Committee Members will find in the **Review** tab general information about the project and the whole proposal will be visible either by clicking on **Actions** and then on **View Application in Split Screen** or by clicking on the **View/Print** button next to **Application Preview**.

For each Application, Committee Members will have to fill in the **Description** section (max 2,000 characters). The description should be a summary of the hypothesis to be tested, the specific aims, and the procedures of the proposed research.

Committee Members will be asked to evaluate and score each proposal based on the following criteria:

#### **Project quality and feasibility**

- **Significance** (max 3,500 characters) – Is the link to rare genetic diseases properly addressed? Is the proposed research original and/or innovative? Does the proposal offer a clearly stated rationale? Will the results of this research fill a gap in knowledge or an unmet need medical need for the disease? If the project is successful, will it improve therapeutic development?
- **Approach** (max 3,500 characters) – Do the preliminary results support the principles to be tested? Are the experimental approaches/methods appropriate to accomplish the specific aims of the project? Is the project feasible and can be completed within the proposed timeframe? Is the budget appropriate? Does the applicant acknowledge potential problem areas and provide alternative plans? For preclinical projects is there a practical pathway to translation? Please highlight overall strengths and weaknesses.

- **PI and team competence** (max 3,500 characters) - Are the PI and Team appropriately trained and well suited to carry out the work proposed? Is the work proposed proportionate to the level of experience of the principal investigator and key personnel (Partners/collaborators)? Does the Team play a significant role in the field of the submitted research project?

### Scores

Score range: from **1.0 (poor)** to **5.0 (outstanding)** by 0.1-unit increments.

| Full Review Scoring Scale |             |   |
|---------------------------|-------------|---|
| Score                     | Value       | Description                                     |
| 4.6 – 5.0                 | Outstanding | Exceptionally strong with only minor weaknesses |
| 4.0 - 4.5                 | Excellent   | Very strong with no major weaknesses            |
| 3.6 - 3.9                 | Good        | Strong but with moderate weaknesses             |
| 2.0 – 3.5                 | Average     | Few strengths and one or more major weaknesses  |
| 1.0 - 1.9                 | Poor        | Numerous major weaknesses                       |

#### Definitions:

**Minor:** easily addressable weakness that does not substantially lessen the value of the project

**Moderate:** weakness that lessens the value of the project

**Major:** weakness that severely limits the value of the project

When all parts of the evaluation form have been completed, the Committee Members will click on **Submit** and the Application will then be listed in the **Submitted Reviews** tab on the Home Page.

Milan, February 18, 2025