## Multi-round Telethon Call for Research projects 2021-2024 <br> ROUND IV

## FULL REVIEW EVALUATION guidelines 2024 - COMMITTEE

The present 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.

## FULL REVIEW INSTRUCTIONS

Applications that will pass the triage phase will undergo full review and the whole application will be evaluated by three Telethon Scientific Committee members (hereon "Reviewers").

REVIEWERS' ROLE - 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).

EVALUATION - The Reviewers are requested to fill in the "Full Application Evaluation Form" available in TETRA - Telethon Projects Managements system portal at https://projects.telethon.it accessible through personal login and password.

In support of their evaluation, Reviewers will be provided with written comments by External Reviewers, who are chosen ad hoc for each Application by Telethon Research Program Managers.

## Written Comments

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

The individual written comment will be anonymously incorporated into a complete review report that will be fed back 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.

Description (max 2,000 characters including spaces)
The primary reviewer only is requested to fill in the description field by summarising the objectives of the study and the hypothesis to be tested. Also, the primary Reviewer will concisely describe the specific aims and procedures of the proposed research.

The Reviewers will be asked to evaluate and score each proposal based on the following criteria:

## Project quality and feasibility

- Significance (max 3,500 characters including spaces) - 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? If the project is successful, will it improve therapeutic development?
- Approach (max 3,500 characters including spaces) - 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 project is there a practical pathway to translation? Please highlight overall strengths and weaknesses.
- PI and team competence (max 3,500 characters including spaces) - Is 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 |  | Value |
| :---: | :--- | :--- |
| Score | 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

Milan, January 31 ${ }^{\text {st }}, 2024$

