



## TELETHON-UILDM CLINICAL PROJECTS – CALL 2024

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### GUIDELINES FOR THE CLINICAL TRIAL EVALUATION COMMITTEE

Fondazione Telethon ETS (“Telethon”) is an Italian charity recognized by the Italian Ministry of Education, Universities and Research, whose mission is to advance biomedical research toward cures for rare genetic diseases. FT funds research projects in Italy through several funding schemes in which projects’ selection is made through a peer-review process ISO9001:2015-certified.

With the present Call, Telethon will devote funds derived from the Patient Organisation UILDM (*Unione Italiana Lotta alla Distrofia Muscolare*) joint fund-raising campaign to **clinical research projects that aim to improve the quality of life of individuals living with a genetic muscular dystrophy condition.**

By the will of UILDM, the focus of this edition will be **clinical research to address the complex medical needs of adults with muscular dystrophy. Studies may also include, but not be limited to, information related to the transition into adulthood.**

Clinical research projects on the aforementioned diseases must address the following research areas:

- Cardiological involvement
- Cognitive and behavioural impairment
- Falls, fractures and bone metabolism
- Natural history studies and outcome measures
- Nutritional needs and bulbar function
- Prognostic or predictive biomarkers

Multicentre and multidisciplinary projects are encouraged. **The direct participation in the project of young investigators as Principal Investigator/Coordinator/Partner is also highly encouraged.**

Awards are contingent upon the availability of funds.

### EVALUATION INSTRUCTIONS

**REVIEWERS’ ROLE** - Each application is reviewed and scored by three Committee members, referred to as “Reviewers”. The **primary** reviewer is responsible for presenting the project during the plenary session. Both **primary and secondary** reviewers are required to provide written comments, while the **tertiary** reviewer is not required to do so (but may provide written comments if they wish).

**EVALUATION** – The Reviewers are asked to complete the “Evaluation Form” available in the *TETRA - Telethon Projects Managements system portal* at <https://projects.telethon.it>. This can be accessed using personal login and password.

Written comments from External Reviewers, who are selected specifically for each application by Telethon Research Program Managers, will be provided to support the evaluation.

### Written Comments

Written comments are crucial for the review process and are used to develop summary statements for the applicants.

Your comments will be directly and anonymously incorporated into a complete Review Report that will be fed back to the Applicant. It is important that your written material is accurate, clear, and free from any derogatory language.

Please note that External Reviewers' written comments will also be included in the review report.

#### **Description** (max 2,000 characters including spaces)

Primary reviewers only are requested to complete the description field. This should summarise the study's objectives and the hypothesis to be tested, as well as concisely describing the specific aims and procedures of the proposed research.

#### **Scientific comments** (max 18,000 characters)

This section should provide a comprehensive and thorough evaluation of the application.

For revised applications only, Reviewers should evaluate the changes made in response to previous critiques and indicate whether the Application has improved compared to the previous submission. The Telethon Review Report of the previously submitted application, along with the Applicant's rebuttal, are available in the *Cover Letter* section of the Application.

- **Scientific Merit** - The Reviewers are required to evaluate the scientific merit of the proposal by analysing its strengths and weaknesses based on the following parameters:
  - Link to eligible diseases: does the proposal address one (or more forms of) muscular dystrophy? Is it targeting the adult life?
  - Significance: Does this study address a significant problem? If the proposed project achieves its aims, how will it advance scientific knowledge on the disease(s) of interest? What will be the impact of these studies on the concepts or methods that drive this field?
  - Originality of science: Does the project employ novel concepts, approaches or methods? Are the aims original and innovative? Does the project challenge existing paradigms or develop new methodologies or technologies?
  - Preliminary results: Does the proof-of-principle information provided adequately support the new principles to be tested in the grant? Are novel tools (or reagents, if applicable) well-characterized?
  - Appropriateness of design and methods: Are the conceptual framework, design, methods, and analyses well-developed, integrated, and appropriate for the project's aims?
  - Feasibility: Does the applicant acknowledge potential problem areas and consider alternative strategies?
  - Safety: Assess the adequacy of the proposed protection for humans to the extent that they could be adversely affected by the proposed project (if any).
- **Impact on patients** - What is the potential of the proposed project to progress towards therapy or to have any other impact on patients' clinical management and/or quality of life? How close is such development expected in time?
- **Comments on Applicant** - Is the investigator appropriately trained and well suited to carry out the proposed work? Is the proposed work proportionate to the experience level of the **Principal Investigator**,

key personnel, and of other Partners (if any)? Is the Lead Applicant a significant player in the submitted research project?

Please note that Fondazione Telethon does not assess the Candidate’s CV based on journal-based metrics, such as Journal Impact Factors. The Fondazione has signed and endorsed the San Francisco Declaration on Research Assessment (DORA, <http://www.ascb.org/dora/>).

- **Comments on Budget Allocation** - Evaluate the appropriateness of the proposed budget in relation to the proposed research. This section should determine whether all items of the requested budget are considered realistic and justified in terms of the research’s aims and methods. Present reasons for any recommended modifications in the amount or duration of support must be presented. Identify any scientific or budgetary overlap with active or pending support.

**Overall evaluation** (max 2,000 characters including spaces)

This section is for the Reviewers to summarize the key reasons for their overall rating, highlighting the relative strengths, weaknesses, and overall final considerations.

**Overall Score**

Scoring scale and rating criteria:

<i><b>SCORE</b></i>	<i><b>VALUE</b></i>	<i><b>RECOMMENDATION</b></i>
4.5 - 5.0	Outstanding	No Concerns - Highest priority for funding
4.0 - 4.4	Excellent	Non substantial issues - Funding is recommended
3.5 - 3.9	Good to Very good	Only few addressable concerns - Funding is deemed appropriate, if funds are available
3.0 - 3.4	Average	Not fundable
2.0 - 2.9	Below average	
1.0 - 1.9	Unacceptable	

Please use the complete scoring range to avoid a clustering of projects within a narrow intermediate range that would make the selection process difficult.