



TELETHON-UILDDM CLINICAL PROJECTS – CALL 2023

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 deriving from the Patient Organisation UILD (Unione Italiana Lotta alla Distrofia Muscolare) joint fund-raising campaign to **clinical research projects aimed at improving quality of life of people living with a genetic muscular dystrophy condition.**

By the will of UILD, the focus of this edition will be **clinical research to address the complex medical needs of people with a muscular dystrophy in their adult life. Studies may also include, but not be limited to, information related to transition into adulthood.**

In particular, clinical research projects on the above diseases have to 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 (hereon “Reviewers”). The **primary** reviewer is responsible for the project’s presentation during the plenary session. **Primary and secondary** reviewers have to provide written comments, whereas **tertiary** one is not requested to (but may provide written comments if he/she wants to).

EVALUATION – The Reviewers are requested to fill in the “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 your review and are critical in developing summary statements for the Applicants.

The individual written comments will be directly and anonymously incorporated into a complete Review Report that will be fed back to the Applicant. It is therefore important that your 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)

Primary reviewers only are requested to fill in the description field. This should summarise the objectives of the study and the hypothesis to be tested, it also concisely describes the specific aims and procedures of the proposed research.

Scientific comments (max 18,000 characters)

This section should present a comprehensive evaluation of the application.

For revised applications only, the Reviewers are asked to provide their evaluation of the changes and responses to the critiques from the previous review, with the indication of whether the Application has been improved comparing to the previous submission. The Telethon Review Report of the previously submitted application together with the Applicant's rebuttal are available within the Application (*Cover Letter* section).

- **Scientific Merit** - The Reviewers are asked to evaluate the overall scientific merit of the proposal by providing an analysis of the strengths and weaknesses on the basis of the following parameters:
 - Link to eligible diseases: is the proposal addressing one (or more forms of) muscular dystrophy? Is it targeting the adult life?
 - Significance: Does this study address an important problem? If the aims of the application are achieved, how will scientific knowledge on the disease(s) of interest be advanced? What will the effect of these studies be 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: Is the proof-of-principle information provided adequately supporting new principles to be tested in the grant? Are novel tools (or reagents, if applicable) well characterised?
 - Appropriateness of design and methods: Are the conceptual framework, design, methods, and analyses adequately developed, well integrated, and appropriate to the aims of the project?
 - Feasibility: Does the applicant acknowledge potential problem areas and consider alternative tactics?
 - Safety: Please also evaluate the adequacy of the proposed protection for humans to the extent they may be adversely affected by the project proposed in the application (if any).
- **Impact on patients** - What is the potential of the proposed project to make progress towards therapy or to provide 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 this work? Is the work proposed proportionate to the level of experience of the **Principal Investigator** and 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 apply assessment of Candidate’s CV based on journal-based metrics, such as Journal Impact Factors. The Fondazione 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 narrative section should establish whether all items of the requested budget are considered realistic and justified in terms of the aims and methods of research. Reasons for each recommended modification in amount or duration of support must be presented. Identify any apparent 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, indicating the relative strengths, weaknesses and overall final considerations.

Overall Score

Scoring scale and rating criteria:

<i>SCORE</i>	<i>VALUE</i>	<i>RECOMMENDATION</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.