



TELETHON-UILDM CALL FOR CLINICAL PROJECTS - 2019

GUIDELINES FOR THE CLINICAL TRIAL EVALUATION COMMITTEE

With this Call, Fondazione Telethon devotes funds deriving from the UILDM (*Unione Italiana Lotta alla Distrofia Muscolare*) fund-raising campaign towards **clinical research projects aimed at improving the quality of life of patients affected by genetic muscle disorders**. Being muscular dystrophies and myopathies the main pathologies addressed by the UILDM, proposals on these diseases will be considered of highest priority.

REVIEWERS' ROLE

Each application is reviewed and scored by three Committee members. The **primary reviewer** is responsible for the project's presentation during the plenary session. Both **primary and secondary reviewers** have to provide written comments, whereas the **tertiary reviewer** is not requested to.

EVALUATION FORM GUIDELINES

Overall Score

Scoring scale and rating criteria:

SCORE	VALUE	RECOMMENDATION
4.5 - 5.0	Outstanding	Highest priority for funding
4.0 - 4.4	Excellent	Funding is recommended
3.5 - 3.9	Good to Very good	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.

Critique

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

Your individual critiques 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.** Therefore, please do not report External Reviewers' sentences in your written comments, as this would result in a duplicated feedback to the Applicant.

Description (max 2,000 characters including spaces)

Summarise the specific aims of the study and the hypothesis to be tested. Concisely describe the objectives and procedures of the proposed research. **Do not make evaluation statements in this section.**

Scientific Merit (max 10,000 characters including spaces)

This section should present a comprehensive evaluation of the application. **Please do not repeat the project description; only evaluation statements should be included.**

For revised applications only, also evaluate progress changes and responses to the critiques from the previous review and indicate whether the application has been improved, is the same as or is worse than the previous submission. You can find the Telethon Review Summary of the previously submitted application, together with the Applicant's rebuttal, in the Cover Letter section.

Evaluate the overall scientific merit of the proposal by providing an analysis of the strengths and weaknesses on the basis of the following parameters:

- Significance: Does this study address an important problem? If the aims of the application are achieved, how will scientific knowledge 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?
- 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?
- Preliminary results: Are proof-of-principle experiments provided, adequately supporting new principles to be tested in the grant? Are novel tools or reagents well-characterized?
- Feasibility/safety: Does the applicant acknowledge potential problem areas and consider alternative tactics? 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).
- Link to genetic diseases: is the proposal addressing a genetic disease? Does the proposal bear the potential to advance knowledge on the disease(s) of interest?

Impact on patients (max 2,000 characters including spaces)

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 in time is such a development envisaged?

Comments on Applicant (max 2,000 characters including spaces)

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 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 endorses the San Francisco Declaration on Research Assessment (DORA, <http://am.ascb.org/dora/>).

Comments on Budget Allocation (max 2,000 characters including spaces)

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.

Evaluation of the Previous Achievements (max 3,000 characters including spaces)

In the case of former grantees only, assess it by expressing your overall evaluation of the scientific results achieved, relative to the grant's original plan, as well as to the continuation of the research in the present proposal.

Overall evaluation (max 2,000 characters including spaces)

Provide the **key reasons** for your overall rating indicating the relative **strengths, weaknesses** and **final considerations** in the specific fields.