



## TELETHON-UILDMD CALL FOR CLINICAL PROJECTS - 2021

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### GUIDELINES FOR THE CLINICAL TRIAL EVALUATION COMMITTEE

With this Call, Fondazione Telethon (in brief: Telethon) will devote funds deriving from the UILDMD (*Unione Italiana Lotta alla Distrofia Muscolare*) fund-raising campaign to **clinical research projects aimed at improving quality of life of people affected by genetic muscular dystrophy**.

Upon request of the UILDMD, this year only studies dedicated to dystrophinopathies and limb girdle muscular dystrophies are allowed.

In particular, clinical research projects on the above diseases may address:

- Cardiology medicine settings
- Cognitive and neuropsychological assessment
- Nutritional evaluation in relation to patients' metabolic status and body type
- Natural history studies and/or epidemiological studies
- Identification of potential modifier genes
- Identification of 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.

### 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

This section should present a comprehensive evaluation of the application.

For revised applications only, the Reviewers will 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** (max 12,000 characters).

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 dystrophinopathy or limb girdle muscular dystrophy?
- 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).
- Previous achievements (dedicated section for former grantees only): did the previous Telethon grant produce relevant results relative to the stated aims?

- **Impact on patients** (max 2,000 characters)

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 (max 2,000 characters)

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://www.ascb.org/dora/>).

### Comments on Budget Allocation (max 2,000 characters)

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:

<b>SCORE</b>	<b>VALUE</b>	<b>RECOMMENDATION</b>
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.