

ANGELINI FOR FUTURE 2021
Supports for Independent Research in Rare
Diseases

GUIDELINES FOR PREPARING AND SUBMITTING THE
PROPOSAL



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Introduction and general information

The study proposal must be written in English. For abbreviations and acronyms not universally known, spell out the term the first time it is used, with the appropriate abbreviation in parentheses; the abbreviation should then be used thereafter.

The text must be single-spaced, not exceeding the character number limitations specified (which include spaces).

Follow the structure of this template when preparing your proposal. It has been designed to ensure that the important aspects of your planned work are presented in a way that will enable the experts to make an effective and accurate assessment.

Project Title and Acronym title

In addition to study title, an acronym title is possible (maximum 10 characters).

Principal Investigator (PI)

Please indicate the name of scientific coordinator and/or applicant for the Research Project for ANGELINI FOR FUTURE 2020 call.

Host Institution

Indicate the name and address of the Institution, which applies. In case of multicentre studies the centre here indicated is the coordinating centre. Please provide a letter of support to the application signed by the Director or official responsible of the institution.

Overview –Abstract

This section shall give an overall description of the project and shall include specific aims and expected outcomes.

Type of Research

Principal investigator must define the therapeutic area, the disorder and the phase of the study (Preclinical or Clinical phase).

Background-Rationale

Please describe an updated review of already available evidence in the relevant literature with reference to disease, available treatment and therapeutic regimen information on which the study is focused. Furthermore, indication of the main hypothesis to be tested should be provided.

Please select the research plan applicable to the type of study chosen. The other fields can be left blank.

Research Plan for Clinical Study

Objectives of the study

The purpose of the study, the primary and secondary objectives according to statistical hypothesis or descriptive statistics shall be indicated.

Study design

Please describe the study design based on the questions, which are the object of the study proposal, phase of clinical trial (if applicable), study organization; if controlled or not controlled; if superiority, equivalence or non-inferiority is expected.

Study population

The characteristics of the study population and the clinical context, in which the study will be conducted, the procedure and the enrollment time shall be described. Criteria and procedures for withdrawing from the study should be specified.

Inclusion criteria and Exclusion criteria

Please indicate in detail inclusion/exclusion criteria.

Intervention

It shall be provided detailed information on treatments for each group (treatment and control if any) including:

- Dose (and dose increase) and dosage form, packaging and labeling of the investigational medicinal product;
- Route of administration;
- Duration of treatment (including number and duration of cycles, if applicable) and the follow-up period;
- Medicines/treatments allowed (including rescue medicines) and not allowed before and/or during the study;
- Procedures for monitoring compliance of the subjects;
- Description of the "stopping rules" or "discontinuation criteria" for each subject, for a part of the study and for the entire study;
- Experimental drug management procedures, including those for placebo and for the comparator drug, if any.

Outcomes and Endpoints

It shall be indicated primary and secondary endpoints and specific outcome measures; the procedure for the detection of the outcomes (with particular attention to the relationship between subjective and objective endpoint assessment and blindness); the rationale for supporting the validity of each surrogate or composite endpoints, if appropriate, and their clinical relevance.

Methods

A description of the measures taken to minimize/avoid bias, including (but not limited to): *Randomization (if applicable)*. The methods used to generate the randomization sequence shall be indicated. Centralized randomization should be preferred; other randomization procedures should be adequately motivated. Include the procedures for the maintenance of randomization code list as well as the procedures for the code breaking and the identification of the therapies assigned to the subjects. A flowchart describing the comparison groups, allocation procedures, details on dose/duration of treatments and patient follow-up can be attached.

Blinding (if applicable). The presence of blinding and its modalities should be described. In particular, specify if blinding involves personnel for treatment administration and/or outcome assessors.

Data collection. It should be indicated the data that will be collected; the tools used for data collection and their validity and reliability; the measures/indicators used; the potential sources of distortion in the retrieval of information regarding study subjects interventions/treatments; the duration and frequency of follow-up; missing data management. When electronic Case Report Form (e-CRF) is used, only validated systems are acceptable that guarantee traceability (e.g. excel spreadsheets do not represent an adequate data recording system). Please include in this section the identification of source data.

Statistical Plan

Statistical hypothesis shall be detailed.

Calculation of the sample size. Please indicate the estimate of sample size and how it is defined. The information required to calculate the sample size include potency, level of significance, incidence in the population under investigation and treatment effect size. The adjustment for other factors that affect the calculation of sample size (e.g. expected compliance rates) should also be reported. For equivalence/non inferiority studies, the largest acceptable difference should be specified.

Statistical Analysis. Please describe the main statistical analyses that will be carried out. The definition of the populations for the main analysis and the probability of error should be indicated. A brief description of statistical techniques, additional methods of analysis, and possible analyses by subgroups should be provided. The main statistical analyses that will be used in the presentation of final results (e.g. final reports, publications) shall be consistent with this section. The planning of each interim analysis (if any) and the predefined study interruption rules shall be clearly indicated. Indicate the estimation of drop out patients and possible impact on study results.

Timing

Provide a tentative sequence or timetable for the project and a GANTT chart of activities.

Research Plan for Pre-Clinical Study

Objectives of the Study

Describe the overall objective(s) that the proposed research is intended to accomplish.

Study description

Describe the experimental plans, including rationale for choice of in vitro cell/tissue assay and/or animal model with a clear study design and details of endpoints being measured.

For in vitro cell assays, provide details on any cell lines that will be used.

For in vivo studies, justify choice of species, animal numbers and group size (e.g. using statistics/power analysis for primary endpoints).

For studies using transgenic mouse, tissue or cells include details of the genetic background with appropriate published references.

Methods

Describe the specific tools, procedures, materials and techniques you plan to use.

Expected Outcomes

Anticipate outcome of results and describe the impact of the results (e.g. progressing this compound as a therapy or increasing understanding of mechanisms in disease).

Timing

Provide a tentative sequence or timetable for the project and a GANTT chart of activities.

References

List of key publications used in your proposal.

Host Institution

Provide administrative details regarding the host institution

Applicant

List the publications considered relevant for the Project. Please note that the submission of *Curriculum vitae* of PI is mandatory.

Collaborators

List collaborators who are actively involved in the project and, as such, describe their contribution to the project.

Budget

The budget must be consistent with the complexity of the study, and should be adequately motivated and detailed. Please complete the budget worksheet. Rows can be inserted in each section. Some fields may not be applicable to your study. Those fields can be left blank.

Equipment and travel costs are not allowed.

Overhead rate should be according to local requirements and not higher than 20 % of the direct costs.

Submitting application

Complete applications must be received at research@incipit-ped.net email address by **March 31st 2021 at 5 pm.**