2020 ELA clinical trial FOA Instructions & Policies

2020
ELA clinical trial
Funding Opportunity
Announcement
(FOA)

A. INSTRUCTIONS

I. SCOPE of RESEARCH

Before starting to work on your application, please make sure the scope of your clinical project fits with the topics required by ELA International:

ELA International represents national ELA associations from different countries that contribute together to the financing of research on leukodystrophies. It is run by parents and /or patients affected by leukodystrophies. Accordingly, ELA International wishes to open a one-time funding opportunity on a program devoted to the conduction of clinical trials in women with X-linked leukodystrophy.

Funding Opportunity Purpose

The goal of the program is to support the clinical development of a therapy in leukodystrophies, the clinical development of products for use in leukodystrophies where no current therapy exists or where the product being developed will be superior to the existing therapy. ELA will provide a grant for a single clinical trial on safety and effectiveness that will either result in, or substantially contribute to, the validation of a therapy in leukodystrophies or market approval of these products. Applicants must include in the application an explanation of how the proposed study will either help support product approval or provide essential data needed for product development.

The purpose of this FOA is to encourage applications for investigator-initiated exploratory clinical trial to ELA International. The trials must address questions within the mission and research interests of ELA International and may evaluate drugs, biologics, and devices, as well as surgical, behavioral and rehabilitation therapies in leukodystrophy patients. Information about the mission and research interests of ELA International can be found at ELA International website http://elainternational.eu/en.

Note the following:

- For purposes of this FOA, the proposed study should be intended to clinically develop the interventions to prevent or treat a leukodystrophy.
- An application involving a clinical experiment that is not directly intended to develop a therapeutic intervention is not suitable for this FOA. This would include, for instance, an experiment where the objective is to elucidate the pathogenesis of the disease or identify potential therapeutic targets for future exploratory trials.

When appropriate, later-stage studies should include randomization and blinding and should yield data that allow a clear go/no-go decision regarding whether the trial should proceed to therapy authorization by the medical regulatory agencies.

Award process

The scientific merit of applications is determined by peer review. A Review Committee will recommend applications for funding based on the scientific merit, together with their judgment of the degree to which proposals are responsive to ELA's mission and research priorities and have potential for high impact.

Submission of a Letter of Intent (LOI) is required to apply for the 2020 ELA clinical trial FOA. To apply, simply:
Download the LOI Application Form online at http://elainternational.eu/en/clinical-trial-FOA.
Return the completed form by email no later than midnight CET Tuesday July 14th, 2020 and by post to ELA Research department no later than midnight CET Tuesday July 21st, 2020.
Eligible projects, according to clinical merit scores, will then receive a detailed application form to be completed. Projects selected by the Review Committee will be notified by November 2020 and invited to submit a full application by February 2021.
NB: All clinical trial applications must obtain regulatory agency authorizations by January 1st, 2021.

Required Application Instructions

It is critical that applicants read and follow all application instructions in the 2020 ELA clinical trial FOA Instructions and Policies (http://elainternational.eu/en/clinical-trial-FOA).
Conformance to all requirements is required and strictly enforced. Applications that do not comply with these instructions may be delayed or not accepted for review.

Letter of Intent submission deadline: July 14th, 2020

Themes proposed in the 2020 ELA clinical trial FOA will focus on clinical trial in the field of women with X-linked leukodystrophies.

Research Topics of Interest includes:

- Clinical trial for women with X-linked leukodystrophies

The trials should address a therapeutic gap in the field of leukodystrophies.
The Applicants must use the platform Leuconnect for the conduct of their study.

Funding Opportunity Description

Funding Instrument:
Grant: A support mechanism providing money to an eligible entity to carry out an approved clinical trial.

Application Types Allowed:
New

Award Budget and Project Period:
Direct costs requested to ELA cannot exceed 500,000 € in total, with a two year-limit in funding period. A preference will be given to shorter trials (1 year trials). For trials lasting more than one year, the grant will be reassessed each year. Joint funding initiatives are encouraged.
In return for the grant allocated, the Applicants will be committed to use the platform Leuconnect for the conduct of their study, to make the Data collected within the framework of the Research freely available to ELA International and academic partners. Co-ownership of the patents and/or financial grant-back in case of valorization or exploitation of the Results shall be agreed upon between ELA International and the grantee.

For more information: elise.vivar@ela-asso.com

II. PREPARING YOUR APPLICATION

A. GENERAL INSTRUCTIONS

Prepare the application using the Word forms provided:
2020 ELA-FOA LOI Application Form (available online at http://elainternational.eu/en/clinical-trial-FOAhttp://elainternational.eu/en/clinical-trial-foa/) and 2020 ELA-FOA Full Application Form. Only eligible projects, according to clinical merit scores, will receive the detailed application form to be completed. No other form will be accepted.
Read and follow the instructions carefully.
The document must be filled out in English *ONLY* and must comply with the format specifications and page limits detailed in this document. Use Arial font size 11 point. Greeks letter are allowed. Same font size applies. Do not reformat the forms or exceed the space provided in the different sections of the grant application. **Failure to comply will result in administrative withdrawal.** Decisions of administrative withdrawal are final and not subject to appeal.

Most of the sections in the application form are self-explanatory.
Each page, but the front page, contains a heading named "Principal Investigator" (PI) where the name of the PI has to be entered.
The page numbering and Table of Contents are set automatically and need to be updated before submission.

2020 ELA clinical trial FOA APPLICATION
Eligibility: The Principal Investigator must have a faculty appointment (position equivalent to assistant professor or higher) in order to be eligible for a grant from ELA International.

B. SPECIFIC INSTRUCTIONS

Cover
Co-financed project
The clinical trial can be co-funded. Indicate if a joint funding is expected by select the appropriate value: Yes, No.

Years of support
The clinical trial can be funded for a maximum of two years. Select the appropriate value: 1 or 2.

Type of Institution
Choose *ONE* of the following types: Public, Private/Non-profit, or Private/Profit.

Abstract (maximum 500 words)
State the general interest of the clinical trial project, the objectives and specific aims. Describe concisely the design and methods for achieving the goals. Describe the rationale and techniques you will use to pursue these goals. Clearly describe the expected results and the relevance to health and therapy for leukodystrophies. Do not exceed the word limit.

Rationale (maximum 500 words)
Describe in maximum 500 words the novelty of your project, its relevance with regards to the topics of the 2020 ELA clinical trial FOA and the potential impact of your project to advance the development of therapies for leukodystrophies.

Lay Summary (maximum one page)
Describe in a plain, lay language the aims of your clinical project, the means to be used to test your hypotheses and the relevance to health & therapy for leukodystrophies. Do not exceed the page limit.
If the grant is awarded to your clinical trial project, the lay summary will become public information. Therefore, do not include proprietary/confidential information.

Synopsis
Please provide the synopsis of the trial.

Overall Global Budget

Use ONLY the Euro currency in the table.

For each year, indicate the projected overall budget and the amount requested to ELA.

% of total cost requested to ELA will be calculated automatically (Grey column). Overall global costs will be calculated automatically (Grey column). Do not modify the table.

Other Sources of Funding

For co-financed project, list other sources, acquired and pending, for the funding of the clinical trial.

Provide one source per line.

Indicate the Name of the person or body providing fund. For institutions, specify if Public, Private/Non-profit, or Private/For Profit. Indicate the amount in euros, if source of funding is active or pending, and the grant date expected for the pending sources.

Add additional lines to the table, as needed.

Notes:

Direct costs requested to ELA cannot exceed 500,000 € in total, with a two years-limit in funding period. Joint funding initiatives are encouraged.

ELA International neither covers overhead/ indirect costs, nor salaries for principal investigators, co-investigators and scientists with permanent or tenure-track positions.

APPENDIX MATERIAL

Do not attach any documents other than those specifically requested.

C. SPECIFIC INSTRUCTIONS for 2020 ELA-FOA Full Application Form

Upon invitation, the following documents must be included in the Full Application in the order listed below:

1. Initial ELA-FOA LOI Application
2. Detailed budget
3. Clinical protocol
4. Informed consent forms (ICFs) and, if applicable, assent form(s)
5. Statistical Analysis Plan (SAP)
6. Clinical and Data Monitoring Plans
7. List of participating clinical sites, pharmacies and laboratories
8. Investigator's Brochure (IB) or equivalent for the intervention, if the intervention is a drug or biologic
9. Material Safety Data Sheets, as appropriate
10. Documentation of availability of interventional agent(s) or device(s) as well as plans and support for acquisition and distribution of interventional agent(s) or device(s)
11. Regulatory Approvals
12. Documentation of availability of eligible subjects at clinical sites, presented in tabular format
13. Milestone Plan. Applicants are required to provide detailed project performance and timeline objectives. The proposed milestones must include achievable goals for the start-up stage, feasibility stage, and completion stage of the project as follows
    - Completion of start-up activities (finalization of protocol, contracting of sites, registration in ClinicalTrials.gov, completion of any final regulatory approvals, etc.)
    - Enrollment of the first subject
    - Enrollment of 25%, 50%, 75% and 100% of the projected recruitment for all study subjects, including women, minorities and children (as appropriate)
    - Completion of data collection time period
    - Completion of primary endpoint and secondary endpoint data analyses
    - Completion of final study report
    - Publication of primary study results
    - Reporting of results in ClinicalTrials.gov
    - Submission of final public use dataset to ELA International
    - Adaptive designs, allowed under this mechanism, should include a pre-specified adaptation plan that allows for clear go/no-go decisions.

Budget Forms

Use ONLY the Euro currency in the tables.

Budget Summary per Year

Complete one table for each year of support requested.

For each category, indicate the overall budget projected for the corresponding year, and the amount requested to ELA.

% of total cost requested to ELA will be calculated automatically (Grey column). Global costs per year will be calculated automatically (Grey column). Do not modify the table.

Budget Justification

Total study administrative cost

List the administrative expenses linked to the clinical trial and provide detailed projected costs related to these expenses.

Total study personnel cost

List the personnel related costs necessary for the conduct of the clinical trial and provide detailed projected costs related to these expenses.

Total patients/participants cost and & Per patient/ participant cost

List the patient related care and treatment expenses involved in the conduct of the clinical trial (drugs, supplies, clinical examinations, visits…) and provide detailed projected costs related to these expenses. Provide details for the total budget and for the cost per patient/ participant. Do not include patient/ participant-related travel cost.

Total patients/participants travel cost & Per patient/ participant travel cost

List the patient travel related costs necessary for the conduct of the clinical trial and provide detailed projected costs related to these expenses. Provide details for the total budget and for the cost per patient/ participant.

Other sources of funding

If some trial costs are to be borne by sources other than ELA, these contributions must be presented in detail in the budget justification.

Global Budget Summary

For each category, i
ndicate the overall budget and the amount requested to ELA.
% of total cost requested to ELA will be calculated automatically (Grey column). Overall global costs will be calculated automatically (Grey column). Do not modify the table.

III. SUBMITTING YOUR APPLICATION

Electronic submission

The electronic ELA-FOA LOI Application must be submitted by midnight CET on **July 14th, 2020** (Paris time) to elise.vivar@ela-asso.com.

Signed hard copy

A hard copy of the pages from the application requiring a signature (and only these) must be submitted to ELA with the required **SIGNATURES AND SEALS** and must arrive by **July 21th, 2020** to: ELA – Research Department - 84, rue d'Hauteville - 75010 Paris, France.

Cover letter

Along with the signed hard copy pages, include a cover letter listing, if applicable, referees (with their contact information) who should not review your application because of conflict of interest. *Mandatory*: give three names of referee with their contact information (excluding your project's collaborators) that you judge qualified to evaluate your project (Yet other experts might be considered to evaluate your application).

IV. GENERAL CONSIDERATIONS

No hard copy pages will be returned.

The application must be complete and accurate at the time of submission. An application is considered complete only when accurate electronic and signed hard copy pages have both been received by their respective deadlines.
Incomplete applications will be administratively withdrawn and not returned to the applicant.
Late applications will not be accepted.

Modifications

The electronic and signed hard copies must match entirely. No modifications are allowed. If changes are being made, the application will be withdrawn. If minor administrative changes are needed after submission (such as budget miscalculations, incorrect address…), the principal investigator needs to contact ELA Scientific Coordinator.

Supplementary material

No supplementary material or updates will be accepted after the deadline. The only exceptions are missing documentation requested by ELA Research Department.

Acknowledgment of receipt

After reception of the complete application by ELA Research Department, the PI, co-investigators (if applicable), and legal grant officer(s) will receive an acknowledgment of receipt to the e-mail addresses entered in the grant application.

V. Application Review Information

Criteria

Reviewers will provide an overall impact score to reflect their assessment of the likelihood for the project to exert a sustained, powerful influence on the clinical field of leukodystrophies.

Scored Review Criteria

Significance
Opportunity of treatment
Duration: A preference will be given to shorter trials

Review and selection process

Applications will be evaluated for clinical merit by the FOA Review Committee, an appropriate group of peers convened by ELA, using the stated review criteria.
Process: Submission of a Letter of Intent (LOI) is required to apply for the 2020 ELA clinical trial FOA. Eligible projects, according to clinical merit scores, will then receive a detailed application form to be completed.

Award Notices

A formal notification will be provided to the applicant organization for successful applications.

VI. SUPPORT

For assistance, contact the Scientific Coordinator of ELA at ELA – Research Department - 84, rue d'Hauteville - 75010 Paris, France.

E-mail: elise.vivar@ela-asso.com

B. 2020 ELA clinical trial FOA GRANT POLICIES

I. GENERAL GUIDELINES

WARNING
The application must be complete and accurate at the time of submission. An application is considered complete only when accurate electronic and signed hard copy have both been received by their respective deadlines.
Incomplete applications will be administratively withdrawn and not returned to the applicant.

I.1. In signing the application, the principal investigator, and legal grant officer (hereinafter referred to as the "Applicants") agree to comply with the ELA International policies.

I.2. On no account does a grant by ELA International constitute an established right.

I.3. Assurances & Ethical Issues
The Applicants and their respective institutions are responsible for ensuring that appropriate authorization and assurance for the protection of human subjects is being contracted.

I.4. Privileged Communication
Material and information provided by the Applicants in the 2020 ELA clinical trial FOA application are considered privileged communication with the exception of the lay summary that will become public information if the proposal is funded.

I.5. Missing reports
Teams awarded grants from ELA International in the past are reminded that reception of missing reports/documents related to past awards, as indicated in the signed grant agreements, is awaited.

I.6. Project funding support
If the application is approved by ELA International, a Funding Support Form will have to be submitted to ELA International along with the grant Agreement. Grant Agreement will ONLY be processed when the Funding Support Form will be received by ELA International.

II. OBLIGATIONS of the APPLICANT & INSTITUTION

II.1. The grant is allocated for the execution of the clinical trial submitted by the Applicants to ELA International (referred to as the "Clinical Trial").

II.2. In return for the grant allocated, the Applicants are committed to:

A. Inform ELA International of a possible interruption of the Clinical Trial,

B. Inform ELA International for approval before any changes need to be made to the Clinical Trial,

C. Submit a detailed progress report to ELA International within a year, and, in all cases, prior to any renewal of the grant and at the end of the Clinical Trial,

D. Submit within a year a lay summary describing the progress of the Clinical Trial to ELA International in view of transparency for the public whose generosity has enabled the funding of the Clinical Trial, under the restriction of confidentiality when some information in the lay summary is likely to be protected by intellectual property.

E. Submit a financial report to ELA International within a year and, in all cases, prior to any renewal of the grant. The financial report will be signed by the manager of the Institution's Accounting Services, and will detail the utilization of the funds awarded under the Grant Agreement.

F. Acknowledge the support of ELA International in all documents to be published and presented about the Clinical Trial using the grant number provided by ELA International.

G. Participate to the ELA Families-Scientists Meeting and the ELA Scientific Congress upon ELA's request.

II.3. All necessary approvals for the conduct of the Clinical Trial must be obtained by the Applicants from their institutions prior to January 1st, 2021 and the start of the Clinical Trial.

II.4. The Applicants commits to inform ELA International of any other grant applications submitted to other funding agencies overlapping partially or entirely with the Clinical Trial when these applications are submitted. In the event the Applicants are awarded a grant overlapping with the clinical Project, they must notify ELA International within one month of receiving the award notification in order to review the financial participation of ELA International to the Clinical Trial and establish the co-financing arrangements.

III. MONITORING

ELA International is entitled, at any time, to ask the Applicants to provide additional information about the Clinical Trial underway. These additional information will be kept confidentially by ELA International.

IV. PUBLICATIONS

ELA International must be informed of any type of publication or communication regarding the

execution, development and results of the present Clinical Trial, or its follow-up.
These publications and communications must, in every case, mention the contribution provided by ELA International to the execution or follow-up of the Clinical Trial.

V. SANCTIONS

ELA International reserves the right to apply adequate sanctions as stipulated in the grant agreement (document available upon request).

VI. AWARD ACTIVATION

The date of award activation will be decided by the Applicants and the Institution but cannot be set prior to May 1st, 2021.

VII. FINANCIAL GUIDELINES

> WARNING
> The legal grant officer(s) must certify the budget figures by signing the budget form and stamping it with the seal of the institution.

VII.1. Grant Agreements & Money transfer

The grant agreements will be issued by ELA International.
The money transfer related to the Applicants' grant will be set up upon reception of two original signed grant agreements issued by ELA International, after validation of these documents by ELA International.
As the funds are allocated to the Institution, the money will be transferred directly to the Institution's bank account. Transfer of funds to intermediaries (like associations or others) will not be accepted.

VII.2. Budget considerations

The Applicants and the Institution are REQUIRED to use the funds of the grant according to the budget lines indicated in the signed grant agreement.
ELA International authorizes transfers between categories (study administrative costs, study personnel costs, patient related costs, and patient travel related costs) without being previously informed only when the transfer represents less than 20 % of the category to be reduced. When the transfer exceeds 20%, a written authorization from ELA International in the form of an amendment modifying the original grant agreement is required beforehand. This written authorization must be granted before the end of the period covered by the active agreement.

VII.3. Financial report

The Institution is required to submit to ELA International by the end of budget period or upon ELA's request a financial report signed by the manager of the institution's accounting services and detailing the utilization of the funds awarded respecting the budget lines indicated in the signed grant agreement.

VII.4. Credit balance

Any funds not used according to the grant agreement or any unspent funds will have to be reimbursed to ELA International.

VII.5. Credit rollover

No credit rollovers will be allowed if at the end of the Clinical Trial the funding has not been fully spent.

VIII. LEGAL DISPUTES

Any and all disputes between ELA International and the Institution arising concerning the validity, interpretation or execution of the Grant Agreement, that could not be settled amicably, shall be submitted to the relevant courts of Luxembourg.

ELA International
Research Department
84, rue d'Hauteville
75010 Paris
France
http://elainternational.eu/en