



Accelerating Therapeutic Discovery

The Alpha-1 Project, Inc.
Letter of Interest and
Full Application Instructions

VERSION 3
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**The Alpha-1 Project, Inc.
2937 SW 27th Avenue
Suite 302
Miami, Florida 33133**

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I. INTRODUCTION

MISSION

The Alpha-1 Project (TAP) will work with patients, academia, pharmaceutical and biotech companies, and public health organizations in the relentless pursuit of cures and therapies for COPD and liver disease caused by Alpha-1 Antitrypsin Deficiency.

SCIENTIFIC AGENDA

TAP will consider all opportunities which have a strong potential to result in a therapeutic or device for Alpha-1, but is specifically interested in:

- Polymer Inhibitors
- Chaperones
- Anti-proteases/anti-inflammatories
- Gene/stem cell therapy
- Devices for treatment, diagnostics and monitoring

The Alpha-1 Project will concentrate on opportunities that will lead to rapid drug discovery.

WHO SHOULD APPLY

Applicants should be commercial entities or entities with the intent to commercialize a product or device in the short term. Academic researchers are encouraged to apply for the Alpha-1 Foundation's various Research Grant programs. Please visit the Alpha-1 Foundation's website www.alpha-1foundation.org.

APPLICATION AND REVIEW

All projects that are supported by TAP are subject to peer review. This review involves both scientific and business criteria provided by the Scientific and Business Advisory Council (SBAC), which is comprised of experts from scientific and business fields.

In order to streamline the review, a two-step application process is required, consisting of a Letter of Interest (LOI) followed by a full application. LOIs are accepted for a May 1 or November 1 deadline. LOI applicants will be notified in approximately 6-8 weeks whether or not to submit a full application. If a LOI is deemed meritorious, the applicant will be invited to submit a full application. Applicants will be notified within 8-12 weeks of receipt of the full application whether or not to proceed with the project. Funding decisions are made at the discretion of the TAP Board of Directors.

II. LETTER OF INTEREST

Note: All documents submitted to the Alpha-1 Project, including letters of interest, agreements, contracts and IRB approval letters must be translated to English if written in another language.

GUIDELINES FOR PREPARING A LETTER OF INTEREST

A LOI may be solicited by TAP or be an unsolicited submission. The LOI must include the following elements in order for TAP to consider the opportunity for investment:

- Goal of project.
- Full description of the science involved in the project.
- A complete summary of the work completed to date and the stage of development.
- Commercialization strategy.
- Summary of financial position including:
 - Money spent to date on this project and expected spending through to completion
 - Cash flow for YTD and current full year forecast, prior full year actual and projection for next year
 - Current Balance Sheet
 - Statement of committed and uncommitted funds
- Desired level of funding and type of participation by TAP.
- Timing of fund disbursement.
- Period of performance.

The LOI must comply with the following core requirements:

- Typed in standard font (12 point), a maximum of six (6) pages in length.
- Name of the Project Director, position title and applicant organization.
- Project Director's mailing address, telephone number and email address.
- Proposed project's title.
- Names of other key personnel.
- Estimated budget (total amount to be requested).
- Description of the proposed research.
- A two (2) page abbreviated Biographical Sketch of the Project Director, including education, past professional activities, relevant peer reviewed publications, and a listing of the members of the Board of Directors.

- Information on current and past support for the proposed project including lenders, equity partners and their share, and any material covenants.

Where to Submit:

Please mail one (1) original and submit one (1) electronic copy in Word format of the completed LOI to:

David Fernandez, Grants Coordinator
The Alpha-1 Project, Inc.
2937 SW 27th Avenue
Suite 302
Miami, Florida 33133, USA
Telephone: 305-567-9888, extension 242
Fax: (305) 567-1317
Email: dfernandez@thealpha-1project.com

III. FULL APPLICATION

GUIDELINES FOR PREPARING A FULL APPLICATION

The full application must not exceed 25 pages and include the following elements:

- Face Page
- Scientific Project Abstract
- Lay Project Abstract
- Key Personnel, Performance Sites, and Resources
- Biographical Sketch of Project Director
- Initial Project Progress Report and Preliminary Data
- Project Design, Method and Expected Results
- Budget and Budget Justification
- Human Subjects and/or Vertebrate Animals (if applicable)
- Literature Cited
- Contractual Arrangements and Consultants

FORMAT SPECIFICATIONS

All sections of the application (e.g., Biographical Sketch, Introduction, if necessary, etc.) must conform to the specifications described below.

When applicable, prepare the full application single-sided and single-spaced. The print must be clear and legible. Use standard size, black letters that can be clearly copied.

If terms or acronyms are not universally known, spell out the term or the acronym the first time it is used, with the appropriate abbreviation in parentheses. The abbreviation may be used thereafter.

Observe type size and format specifications. The application must be clear, legible, and conform to the following four specifications:

1. The height of the letters must not be smaller than 12 point; Arial, Garamond, Times New Roman or Verdana are TAP's suggested fonts.
2. Type density, including characters and spaces, must be no more than 15 characters per inch (cpi);
3. No more than 6 lines of type within a vertical inch;
4. Margins, in all directions, must be at least ½ inch.

Figures, charts, tables, figure legends, and footnotes may be smaller in size but must be legible.

SPECIFIC INSTRUCTIONS FOR PREPARING A FULL APPLICATION

1. Face Page (1 page): The Face Page of the full application must include the following elements:

- a. Title of Project:** Choose a title that is specifically descriptive, rather than general.
- b. Project Director:** Provide the name of the single person responsible for the scientific and technical direction of the proposed research. Provide terminal professional degree or other credentials.
- c. Position Title:** Provide the academic or professional title of the Project Director.
- d. Contact Information:** Provide complete information (including room number, building, and street address) necessary for postal delivery. All written communications with the Principal Investigator will use this address. Applicants are required to provide their e-mail addresses, phone number(s), and fax number(s).
- e. Human Subjects (Yes or No):** Indicate whether or not the proposed research will involve the use of Human Subjects.

Should your proposal require the use of an IRB approval, the approval letter must be received by the Alpha-1 Project prior to the start of the grant. IRB approval is not required at the time of the project submission.

- f. Vertebrate Animals (Yes or No):** Indicate whether or not the proposed research will involve the use of animals.
- g. Proposed Period of Support:** Do not use dates.
- h. Total Costs Requested for the Entire Period of Support:** Provide the amount requested for the entire period of support in U.S. dollars.
- i. Total Costs Requested for Initial Budget Period:** Provide the amount requested for the initial budget period in U.S. dollars.
- j. Applicant Organization:** Provide the name and address of the organization or institution that will be legally and financially responsible for the conduct of activities supported by TAP.
- k. Official Signing for Applicant Organization:** Provide the name of the individual who is legally authorized to execute contracts on behalf of the applicant organization and will assume the obligations imposed by Federal regulations.
- l. Project Director Assurance:** An original signature, in ink, is required. Date of signature must be included.

2. Scientific Project Abstract (1 page)

3. Lay Project Abstract (1 page)

4. Key Personnel, Performance Site(s), and Resources (1 page):

Performance Site(s): Indicate where the work described in the Research Plan will be conducted. If there is more than one performance site, list all the sites, including foreign sites. One of the sites indicated must be the applicant organization or be identified as off-site. State if a consortium-contractual arrangement is involved with one or more collaborating organizations for the conduct of a portion of the work described in the Research Plan. If a performance site is participating in research using human subjects, it is the responsibility of the applicant organization to assure that the performance site

complies with the regulations in 45 CFR Part 46. A letter of support is required from each performance site named in the full application.

Key Personnel: Key personnel are defined as individuals who substantively contribute to the scientific development or execution of the project, whether or not salaries are requested. Consultants should also be included if they meet the definition of key personnel. A Biographical Sketch must be submitted for each person who is listed as key personnel, including consultants.

Resources: Specify the facilities to be used for the conduct of the proposed research. Indicate the performance sites and describe capacities, pertinent capabilities, relative proximity, and extent of availability to the project.

5. Biographical Sketch of Project Director (1-2 pages): Provide a Biographical Sketch of the Project Director, including education, past professional activities, and relevant peer reviewed publications.

6. Initial Project Progress Report and Preliminary Data (not to exceed 2 pages): This section should reflect the state of the art in the proposed area of investigation, the rationale for the proposed project, and the prior work on this project leading up to this proposal. This should also include prior publications by the applicant relating to the proposed project.

7. Project Design, Method, and Expected Results (not to exceed 10 pages, including tables and figures): Describe the research design and the procedures to be used to accomplish the specific aims of the project. Provide information on how the data will be collected, analyzed, and interpreted, as well as the data-sharing plan as appropriate. Describe any new methodology and its advantage over existing methodologies. Discuss the potential risks, difficulties and limitations of the proposed procedures and alternative approaches to achieve the aims. As part of this section, provide a tentative sequence or timetable for the project. Point out any procedures, situations, or materials that may be hazardous to personnel and the precautions to be exercised.

This section should also include milestones and expected timing of progress reports during the proposed project. This information will be important in negotiating the disbursement of funding.

8. Budget and Budget Justification (not to exceed 1 pages): The budget should be separated into the initial project period and total project period, in-line with the proposed milestones and progress reports. The

budget should be sufficiently detailed to permit an assessment of its key elements. This includes costs for salaries (if applicable), supplies, animal maintenance (if applicable), Patient Volunteer and IRB costs (if applicable), equipment, consultant costs, and contractual costs.

The budget should be followed by an explanation of the listed expenses.

9. Intellectual Property (1 page): Are there IPs involved? Please describe the ownership of the IP. If other parties are owners of the IP, please list them, and describe arrangement

10. Commercialization (1 page). Please describe how the product will be commercialized, including:

- Market size in patients and/or dollars
- Competition. Include current competitors, potential entrants, and substitute products
- Entry strategy
- Potential access issues and mitigation. For example, managed care
- Potential reimbursement issues and mitigation
- Other major commercialization risks

11. Financial Statements. Please include the audited financial statements for the last 2 years, including:

- Income Statement
- Balance Sheet
- Statement of Cash flow

Include unaudited Quarterly Statements if the financial position has changed materially.

12. Board of Directors (1 page). Please list your Board of Directors if applicable

- Market size in patients and/or dollars
- Competition. Include current competitors, potential entrants, and substitute products
- Entry strategy
- Potential access issues and mitigation. For example, managed care

13. Project timeline and major milestones (1 page). Please the project timeline and major milestones.

14. Human Subjects (if applicable): If you answered "Yes" on the Face Page of the full application, create a section heading entitled, "Human Subjects Research". In the Human Subjects Research section, applicants must address the involvement of human subjects and protections from

research risk relating to their participation in the proposed project. The key elements of the required information pertaining to the use of Human Subjects can be found on The Alpha-1 Foundation's website (www.alpha-1foundation.org), specifically, in the instructions for in-cycle applications.

15. Vertebrate Animals (if applicable): If you have answered "Yes" on the Face Page of the full application, create a section heading entitled "Vertebrate Animals". In the Vertebrate Animals section, applicants are required to address the following five points. In addition, when research involving vertebrate animals will take place at collaborating site(s) or other performance site(s) provide this information before discussing the five points. Although no specific page limitation applies to this section of the application, be succinct.

1. Provide a detailed description of the proposed use of the animals in the work outlined in the Research Design and Methods section. Identify the species, strains, ages, sex, and numbers of animals to be used in the proposed work.
2. Justify the use of animals, the choice of species, and the numbers to be used. If animals are in short supply, costly, or to be used in large numbers, provide an additional rationale for their selection and numbers.
3. Provide information on the veterinary care of the animals involved.
4. Describe the procedures for ensuring that discomfort, distress, pain, and injury will be limited to that which is unavoidable in the conduct of scientifically sound research. Describe the use of analgesic, anesthetic, and tranquilizing drugs and/or comfortable restraining devices, where appropriate, to minimize discomfort, distress, pain, and injury.
5. Describe any method of euthanasia to be used and the reasons for its selection. State whether this method is consistent with the recommendations of the panel on Euthanasia of the American Veterinary Medical Association. If not, present a justification for not following the recommendations.

16. Literature Cited: Each reference must include the title, names of all authors, book or journal, volume number, page numbers, and year of publication. The reference should be limited to relevant and current literature. While there is not a page limitation, it is important to be concise and to select only those literature references pertinent to the proposed research.

17. Contractual Arrangements and Consultants: Explain the administrative arrangements between the applicant organization and the contractual organization. If contractual activities represent a significant portion of the overall project, explain why the applicant organization rather than the contractual organization should be the awardee.

Where to Submit:

Please mail one (1) original and submit one (1) electronic copy of the completed application to:

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2937 SW 27th Avenue
Suite 302
Miami, Florida 33133, USA
Telephone: 305-567-9888, extension 242
Fax: (305) 567-1317
Email: dfernandez@thealpha-1project.com

Electronic Submission of a Full Application:

An electronic application must be sent as a Word document with all of the required content. Zip files, rich text files or any other format will not be accepted. Should you be unable to submit an electronic application as a Word file, please contact the Alpha-1 Project's Grants Coordinator, David Fernandez, at dfernandez@thealpha-1project.com

Inquires and Interactions:

For questions regarding the TAP's pre- and post- award processes, please contact:

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