



2023 GRANT APPLICATION GUIDELINES

Application deadline: June 16, 2023, at 11:59 p.m. PT

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2023 Grant Instructions

ABOUT EMILY'S ENTOURAGE:

Emily's Entourage (EE) is an innovative 501(c)3 foundation that accelerates research for the final 10% of the cystic fibrosis (CF) community that does not benefit from CFTR modulators, including those with rare and nonsense mutation. Since 2011, EE has awarded millions of dollars in [research grants](#), securing over \$42.8 million in follow-on funding; launched a now-acquired CF gene therapy company; and developed a clinical trial matchmaking program to speed clinical trial recruitment. The organization has been featured in national media, including The New York Times, STAT, CNN, People, and more.

ABOUT CYSTIC FIBROSIS:

CF is caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. Over the past ten years, significant progress has been made with the approvals of CFTR modulator therapies that benefit roughly 90% of individuals with CF with the most common CFTR mutations. Those in the final 10% of the CF population do not benefit from the currently available CFTR modulator therapies.

KEY OBJECTIVES:

EE is pleased to announce the launch of **Emily's Entourage's Gene-based Therapy Initiative**. The objective of this initiative is to fund promising therapies and drug-delivery mechanisms that have the potential to accelerate the development of gene-based therapies for people with CF. Potential areas of interest include but are not limited to the following:

- Gene Editing — Gene editing has the potential to 'fix' mutations in the CFTR gene and represents a promising avenue of research with therapeutic potential, especially for those with rare and nonsense mutations.
- Exogenous Delivery of Full-length CFTR Gene — Inserting a full-length, wild-type CFTR gene to compensate for mutant alleles could restore CFTR levels and functions.
- RNA Editing — RNA editing refers to post-transcription modification of RNA molecules to make changes to specific nucleotide sequences. These changes may include insertions, deletions, and base substitutions.

Emily's Entourage | Fast Breakthroughs for Cystic Fibrosis

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- Non-viral vectors for DNA and RNA delivery — Given the recent advances in novel encapsulation techniques and formulations with carrier particles and customized chemical modifications of nanoparticles, we are focusing our initial efforts to derisk non-viral delivery platforms for genetic therapies.

GRANT PROGRAM OVERVIEW:

Investigators are expected to have a good track record of peer reviewed publications and extramural funding. Awards are set at **\$100,000 per annum** (direct costs) and, typically, funding will last for a **total of two years**. Awards are associated with additional indirect costs set at 10% such that total payments per annum will be \$110,000. Funding will be allocated at 6 month intervals with payments of \$50,000 released by EE, subject to sufficient progress being accomplished. Acceptance of funding requires submission of PROGRESS REPORTS that will be used to assess research progression. Continued financial support is contingent upon satisfactory completion of proposed research objectives delineated in the 'MILESTONES' section of GRANT APPLICATIONS. The first PROGRESS REPORT will be requested after 5 months of funding, and thereafter at 6 month intervals. Acceptance of funding will require execution of the EMILY'S ENTOURAGE GRANT CONTRACT by an authorized representative of the grantee institution.

APPLICANT ELIGIBILITY CRITERIA:

Applicants must have:

- Faculty appointment at an academic institution
- PhD, MD or DO
- There are no restrictions on citizenship or geography
- No funding from other sources for the project for which they are applying to EE
- Track record of publication and funding

EVALUATION CRITERIA:

Competitive proposals will demonstrate:

- Potential to have a major scientific impact for individuals in the final 10% of the CF population that do not benefit from currently available, including those with rare and nonsense mutations.
- Innovation
- Objectives that will enable therapeutic advances



- Feasibility (for instance in the form of preliminary data, or research conducted in the Principal Investigator's laboratory)
- Milestones that are clearly and logically delineated

We strongly encourage collaborative research amongst investigators with experience in gene-targeting strategies within or outside of the CF field.

REVIEW PROCESS:

- Applications will be reviewed internally and by external reviewers prior to consideration by members of the EE Scientific and Leadership Team
- Following preliminary review, additional information may be requested from applicants

KEY DATES:

- 2023 global call for proposals opens on March 20, 2023 and closes on June 16, 2023.
- Applicants will be notified in August if they are selected to move to the next round.
- Those applicants that move to the next round will have the opportunity to address concerns raised by reviewers.
- Projected start date is January 2024.

APPLICATION INSTRUCTIONS:

Provision of the following components is required to complete the GRANT APPLICATION process. All components should be compiled and submitted as a single pdf. In addition, the IMPACT STATEMENT and LAY ABSTRACT (described below) should be provided as a single .docx document. Finalized grant packages should be returned on or before the closing date to Chandra Ghose PhD, CSO, Emily's Entourage (research@emilysentourage.org).

SPECIFIC AIMS: *(Page limit: one page)*

Please provide a project title and include an overview of the proposed research project with proposed objectives, and rationale for the studies described. Also include explicit details of each SPECIFIC AIM that will be addressed to achieve the proposed project goals.

RESEARCH PLAN: *(Page limit: 2 pages)*

The **RESEARCH PLAN** should detail information related to the project including:

- **SIGNIFICANCE** – describe the relevant background for the current research plan



- *INNOVATION* – discuss how the proposed research challenges or advances current knowledge, or introduces novel concepts, approaches or technology
- *APPROACH* – detail methodologies to address the proposed research objectives

It is strongly recommended that the *APPROACH* section includes any pertinent preliminary data. Any cited literature in the GRANT APPLICATION should be included in additional pages using a numbered format.

In terms of institutional compliance, the *RESEARCH PLAN* should explicitly state, as appropriate, that the proposed research requires use of animals and / or human subjects. As necessary, an IACUC approval letter or note if approval is pending, and / or an IRB approval letter or note if approval is pending, should be included in an *APPENDIX*. Otherwise, please note in the *RESEARCH PLAN* that IACUC and / or IRB approval are not required.

MILESTONES: (Page limit: 1 page)

Provide a schematic representation and written description of the proposed project progression based on 6 month intervals. As stated above, continued financial support is contingent on submission of PROGRESS REPORTS and satisfactory completion of proposed research objectives specifically detailed in the MILESTONES section. In the event that MILESTONES are not robust, quantifiable and described in sufficient detail, EE may request resubmission of a revised MILESTONES section prior to making a funding decision. EE reserves the right to terminate funding if there is a lack of adequate scientific progress during any funded time period. The described MILESTONES will play a key role in the assessment of project progression.



BUDGET: (Page limit: 1 page)

Please submit a budget and justification using [this Excel spreadsheet](#) (**this spreadsheet is view only, please be sure to create and work off your own copy of the spreadsheet**) for each of the two years of funding. Indirect costs are set at 10%, which will be included in addition to the \$100,000 annual award (total award \$110,000 per year). The budget justification should explicitly state that overlapping funds do not support the proposed studies.

IMPACT STATEMENT: (Page limit: half page)

Describe how the proposed research will lead to new or improved therapeutic approaches for individuals in the final 10% of the CF population that do not benefit from currently available, including those with rare and nonsense mutations.

LAY ABSTRACT: (Page limit: half page provided on the same page as the IMPACT STATEMENT)

Summarize the research project in a manner that the layperson could appreciate. As described in the **EMILY'S ENTOURAGE GRANT CONTRACT**, information provided in the LAY ABSTRACT will be used and potentially published via formats such as the EE website or social media accounts, at the discretion of EE.

NIH BIOSKETCH(ES):

Please provide an NIH format Biosketch for Principal Investigator(s) associated with the application. In the RESEARCH SUPPORT section, please include all current and pending grant applications, and include an explicit statement that current funding does not directly overlap with funding for the proposed research to be supported by EE.

APPENDIX:

A brief appendix is allowed for information pertinent to the GRANT APPLICATION, including accepted but not yet published manuscripts, additional supplementary experimental results, and similar information. In addition, IACUC and / or IRB approval, and all other pertinent material, should be included in the APPENDIX. The following may be added to the Appendix -

RESOURCE SHARING:

In the event that a resource is generated that could enable development of novel CF therapies for individuals in the final 10% of the CF population that do not benefit from currently available (for instance, cell lines, murine models, etc.), information should be forwarded to



research@emilysentourage.org for inclusion on the EE resource webpage:
<https://www.emilysentourage.org/research-resources/>.

COLLABORATORS AND/OR CONSULTANTS:

Include any appropriate letter from all individuals confirming their involvement in the proposed project.

FORMATTING INSTRUCTIONS:

- All pages of the application should include a header with the name of the Principal Investigator at the top right hand corner, and a footer with a page number in the center of the page
- Application formatting should follow NIH guidelines using 11pt Arial font with a minimum of 0.5" margins
- Any tables or figures should be embedded in the body of the Application and not included in the APPENDIX