Thalassemia Foundation of America
Formerly Cooley's Anemia Foundation

2027-2028 CLINICAL TRIALS IN THALASSEMIA CELL AND GENE THERAPY

The Thalassemia Foundation of America invites national and international applicants to apply for grants to facilitate clinical trials in Cell and Gene Therapy to advance a cure for thalassemia. Both phase I (safety) and phase II (efficacy) trials are eligible for support. Applications that address *in vivo* gene therapy approaches and/or reducing toxicity of gene therapy are encouraged.

FUNDING AND CANDIDATE ELIGIBILITY

Applicants should state explicitly the status of their proposed trials; for example, whether the trial has been approved by the Food and Drug Administration (or equivalent processes at non-US sites) or whether review meetings at regulatory agencies have been scheduled.

Studies proposed for this RFA must be accompanied by a realistic timeline of progress to human trials. The Foundation recognizes that this timeline can be long. While the funds may be used toward any aspect of launching a human gene therapy trial, the Foundation will request careful documentation of other support of the investigator(s) and the project, and a thorough justification of the allocation of costs to various support mechanisms. This will best enable reviewers to assess feasibility of the proposed project. Examples of expenses allowed will include:

- Clinical-grade vector production and quality control
- Research subject assessments
- Late-preclinical studies and *ex vivo* studies on research subjects required for safety and efficacy assessments (including, but not limited to, evaluation of vector genome integration events, studies of transduction efficiency, studies of globin production by transduced hematopoietic stem cells from subjects)
- Patient care costs directly related to the study
- Research assistant or research nursing support.

Assurance must be given that adequate clinical and other facilities (including, where applicable, laboratory and vector production facilities) exist and are available to conduct the research project and, in case of investigations involving human subjects, a full human subject protection plan should be included as an appendix.

Total funding of up to $75,000 per year will be available (including indirect costs of up to 10%). With satisfactory evidence of progress and availability of funding, grants may be renewed for a second year. **Excluded from funding in this grant are lodging and other personal costs for research subjects to come to a treatment center**.

Support for investigator effort may not exceed 10% of the current NIH salary cap.
Animal studies, if any, must be directly relevant to the specific proposed clinical trial.

The research may not be conducted at a for-profit laboratory.

GUIDELINES FOR COMPLETING THE APPLICATION

COVER PAGE

Please complete and attach the form provided on page 4.

NON-TECHNICAL ABSTRACT *(Not to exceed 250 words)*

It is requested that in presenting your abstract you use language easily understandable by a non-technical reader.

PROPOSAL *(Sections I to III should not exceed 10 pages)*

Appendices for clinical trials materials are allowed, but the relevant information for review of the proposal should be within this limit.

I. RATIONALE AND AIMS *(1 page)*
Explain the research program that has led to the development of the proposed clinical/preclinical trial activities. Include a brief overview of the experimental design

II. REVIEW OF LITERATURE and PRELIMINARY STUDIES *(No more than 2 pages)*
Present a coherent view of the present state of research in the field, including contributions of the investigator.

III. DETAILED RESEARCH PROPOSAL *(No more than 7 pages)*

- Describe the proposed study, including methods and analyses to be used to accomplish the specific aims
- Discussion of potential difficulties and limitations and include plans for alternative strategies if initial approaches are unsuccessful
- Figures should be included in the proposal
- A projected timeline should be included
- The clinical trial protocol should be appended to the application, but the application itself should focus on a summary of the trial design and the activities to be supported by the funds provided by TFA.

IV. REFERENCES *(Not included in page count)*

V. FACILITIES AVAILABLE
Please use a standard NIH Facilities and Other Resources page, with continuation sheets as necessary for clinical and other facilities.

VI. OTHER SUPPORT
Please list prior titles, amount per year and duration of all research support for the applicant including pending applications, whether related to this proposal or not.

In addition, detail the support presently available or planned for the proposed clinical trial (which may come from institutional funds as opposed to grants typically listed on “other support” pages).

VII. HUMAN SUBJECTS

Explain the risk, risk-benefit ratio, and methods of obtaining informed consent and of preserving confidentiality. If the funds are proposed to support the human studies themselves, submit evidence of approval of the project by the committee for the protection of human subjects (Institutional Review Board) of the sponsoring institution. This section of the application may be taken from the full protocol directly, but it should address these issues.

For drug studies, include documentation of FDA approval for use of an investigational new drug (IND) if applicable.

VIII. BIOGRAPHICAL SKETCH

Submit a biographical sketch using SciENcv to prepare the form.

IX. BUDGET FOR PERIOD July 1, 2027 to June 30, 2028

Support is available for personnel, equipment, supplies, travel, and clinical trials costs (patient-care and non-patient-care).

- **A.** Salary and Fringe Benefits at the typical institutional rate (Investigator salary not to exceed 10% of NIH cap); other personnel may be supported at higher levels, if required to complete the project.
- **B.** Capital equipment over $5,000 (non-overhead bearing).
- **C.** Supplies necessary for the conduct of the study.
- **D.** Patient Care costs
- **E.** Travel – please justify carefully. Patient travel/lodging during trials is not allowed.
- **F.** OTHER - For example, non-patient care clinical development costs, e.g. vector development
- **G.** Indirect Costs not to exceed 10%
- **H.** Total (not to exceed $75,000)

X. AGREEMENTS

Please attach completed page 5.

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COVER PAGE

Applicant Information

Research Proposal Title

Applicant Name	**Applicant Title**

Mailing Address

Telephone	**Email**

Disbursement Information

Institution Contact Name	**Department**

Check Payable to	**Institution's Federal Identification Number**

Mailing Address

AGREEMENT

I agree with the policies of the Thalassemia Foundation of America concerning this RFA. I certify that I have appropriate facilities to complete the proposed research. Subsequent publications will acknowledge funding by the Thalassemia Foundation of America.

Applicant Name	**Signature**

Additional Signatures

Chairman or Director Name	**Department**

Signature	**Telephone**

Administrative Officer Name	**Telephone**

Signature

Financial Officer Name	**Telephone**

Signature

Approved Human Research Committee

Chairman Name	**Date**

Signature
