

Instructions for Research Proposal Submission 2011 Gaucher Generation Grant Program

Gaucher Generation Program Overview

The Gaucher Generation Program distributes annual grants on a competitive basis to scientists and clinicians engaged in innovative Gaucher Disease research. The Program is intended to stimulate and support the advancement of medical knowledge and practice in Gaucher Disease through innovative investigator-initiated research. It represents Genzyme's ongoing commitment to enhancing the care of Gaucher patients and supporting the Gaucher community. The ultimate goal of the program is to improve the lives of Gaucher patients.

For more details on the Gaucher Generation Program, including information on award amounts, application procedures, and timelines, please visit the Gaucher Generation website at www.gauchergeneration.com.

Research Proposal Overview

The Research Proposal is the second submission in the Gaucher Generation grant application process. Not to exceed 13 pages (excluding requested attachments), the Research Proposal should give a detailed explanation of the intended research objectives and approach. An in-depth description of a distinct and focused research hypothesis, methods, procedures, and research techniques, as well as a clear discussion of potential outcomes, should be included.

The proposal must be in English, single-spaced, single-sided, and written in a 10- or 12-point font in black type. If the proposal includes any unusual abbreviations, please add a page listing abbreviations after the summary page (not included in total page count).

Research Proposal Contents

The Research Proposal should include the information outlined below.

I. Cover Page (1 page):

The cover page should include:

- Name
- Title
- Institution
- Address
- Telephone number
- Email address
- Proposal title

Please do NOT provide these identifiers on any other pages of the proposal as all other pages will be blinded and coded for committee review.

II. Proposal Summary (1 page):

The proposal summary should open with a one-sentence statement explaining the overall research. It should then go on to provide an overview of the proposed research. Applicants may re-submit their Letter of Intent, if so desired, as the rest of the summary. Please do not include institution and investigator names on the summary page as it will be blinded and coded for review.

The summary should include:

- Grant type (NIA) New Investigator Award or (SIA) Senior Investigator Award
- Research rationale
- Hypothesis
- Objectives
- Research plan
- Plan for recruitment and retention of subjects
- Expected outcome
- Estimated duration
- Estimated costs

III. Research Plan (up to 8 pages)

Background

The background section should encompass a focused review of the literature, examining the published studies both supportive of and contrary to the hypothesis. The review should critically evaluate existing knowledge and articulate the logic and clinical relevance of the hypothesis. It should be thorough and should demonstrate complete awareness and understanding by the principal investigator of existing research.

The background section should also explain the value, clinical relevance, and any potential contributions that might be achieved through the proposed research. Significance of the study is a judgment of the value of the research, that is, of the likelihood that the research will result in new knowledge or advances within Gaucher disease and that the research will result in a beneficial impact on the lives of Gaucher disease patients.

Finally, this section should include the principal investigator's research experience and published work relevant to the project to establish competence for conducting the research.

Methods

The methods section should describe in detail the proposed research, study design, methodology, and specific techniques (e.g., statistical methods, instrumentation). This section should also discuss the available facilities and personnel needed for conducting the research and any additional support staff, facilities, access to patients, equipment, supplies, or other support required. When the research proposal deals with a very innovative hypothesis, some preliminary data should be included.

Other sources of research financial support should be listed and copies of any pending grants should be included in the proposal. Collaborators should submit a letter discussing their familiarity with the proposed research topic and their willingness to participate. (These letters will not be counted toward the 8-page limit of the research plan section.)

IV. Budget (1 page)

The proposal must provide details of the proposed expenses to the grant:

- Personnel: No salary for faculty will be allowed; only technical salary will be allowed and capped at 30% of the total grant
- Laboratory (including equipment and supplies)
- Patients
- Medications, if necessary
- Institutional overhead which should not exceed 15% of the total grant amount
- The cost of a non-refundable round-trip ticket in economy class to attend a yearly scientific meeting

The total budget per year must not exceed US \$75,000 for the New Investigator Award (NIA) or \$100,000 for the Senior Investigator Award (SIA).

V. Ethics Approval (1 page)

Approval by the Institutional Animal Care and Use Committee (IACUC) for protocols using animal subjects and by the Institutional Review Board (IRB) or Ethics Committee (EC) for protocols using human subjects must be pending or received at the time of proposal. Submit either a copy of the approval or a copy of the submission. Final approval by the IACUC, IRB, or EC is required prior to initiation of grant payment.

VI. Attachments

Attachments should include:

- Bibliography
- Detailed curriculum vitae of the principal investigator
- Short curriculum vitae of potential collaborators
- Description of the facility or institution where the principal investigator will conduct the research (2-page maximum)

Review Criteria

An independent committee of Gaucher Disease experts will assess all Letters of Intent and Research Proposals.

In addition to the scientific merit of the proposed research, the review committee considers the following criteria when evaluating submissions:

- Adequacy of the experimental design to answer the proposed question of interest
- Feasibility of the proposed study design, budget, and duration
- Clinical importance and potential impact on patients
- Innovation of the proposal
- Qualifications and experience of the principal investigator for the proposed research

To download a sample evaluation form please visit www.gauchergeneration.com.

Research Proposal Application Submission Deadline

The deadline for receipt of Research Proposals at Genzyme is July 1, 2011.

Submission Instructions

Research Proposals should be emailed to Gauchergeneration@genzyme.com. All documentation must reach Genzyme by July 1, 2011.

The proposal may also be submitted via email, on CD-ROM, or memory stick to:

Genzyme Corporation
Gaucher Generation Program
ATTN: Kathleen Collins, Grant Program Coordinator
500 Kendall Street
8th floor
Cambridge, MA 02142 USA

Please confirm receipt of your submission via e-mail to gauchergeneration@genzyme.com. The Grant Program Coordinator will respond to confirm receipt of all Research Proposals.

If you do not receive confirmation receipt of your proposal within two business day, please contact the Generations Coordinator for further assistance.

Gaucher Generation Program Contact Information

For questions regarding the Gaucher Generation program or grant application process, please email gauchergeneration@genzyme.com. You may also call within the US (800) 745-4447, x16370, or + (617) 768-6370 outside the US, and speak with Kathleen Collins, Grant Program Coordinator.