

Alagille Syndrome Alliance

Collaborative Scientific Research Grant Proposal Guidelines

Thank you for your interest in applying for a research grant from the Alagille Syndrome Alliance (ALGSA: www.alagille.org). These guidelines provide a brief introduction to the goals and policies of the ALGSA, and list the specific information required when submitting a proposal.

I. Statement of Research Grant Policy and Procedures

The purpose of the ALGSA is to advocate for better treatments and a cure for those with Alagille Syndrome (ALGS). The ALGSA is also dedicated to increasing public awareness of ALGS, supporting research efforts on behalf of the ALGS community, and educating families, friends and healthcare providers about ALGS.

ALGS is a rare genetic condition affecting about one in 30,000 individuals. Both its symptoms and severity are highly variable and substantial research is needed to understand its complexities. For this reason, the ALGSA awards grants to applicants who seek to conduct basic, pre-clinical, translational, and clinical research projects that investigate all aspects of ALGS. The ALGSA is particularly interested in funding research projects that have the potential to accomplish one or more of the following:

- Identify novel therapies that may significantly improve the quality of life of individuals with ALGS;
- Address an important or novel research question related to ALGS;
- Investigate an aspect of ALGS that is less well researched or is a promising new area for research;
- Promote and encourage ALGS research in young investigators;
- Complement the goals and objectives of the NIDDK (National Institute of Diabetes and Digestive and Kidney Diseases) funded pediatric liver disease consortium ChiLDReN (Childhood Liver Disease Research and Education Network; www.childrennetwork.org) and make use of clinical data available to the network;
- Produce preliminary data that would facilitate broader or more substantial funding from agencies, foundations, or corporations in the future;
- Discover a cure for ALGS.

Proposed project must have specific relevance to ALGS, and show promise for contributing to the scientific advancement in the field of study. Proposals that have little or no relevance to ALGS will not be considered for funding. The ALGSA strongly encourages interdisciplinary collaboration and the sharing of resources as a means of accelerating progress in ALGS research. ALGSA endorses the principles of the Association of American Medical Colleges (AAMC) report, “The Maintenance of High Ethical Standards in the Conduct of Research.”

II. Applicant Eligibility

To be eligible to apply for research grant funding from the ALGSA, individuals or teams of Applicants must demonstrate the following:

- A. Applicant(s) must have a Ph.D., M.D., or equivalent degree;
- B. Applicant(s) must currently hold a full-time position at any level (post-doctoral, research scientist, assistant/associate/full professor, etc.) at an academic institution or in the pharmaceutical/biotechnology industry;
- C. Applicant(s) will be considered from both the United States and internationally.

III. Funding Guidelines and Limitations

- A. Funding is limited to \$150,000 USD for one (2) years; \$75,000 payable at the start of year one; \$75,000 payable at the start of year 2. The following will not be funded by the ALGSA:
 - Overhead or indirect costs;
 - Salaries or stipends for students;
 - General institutional expenses;
 - General fundraising campaign expenses such as dinners and mass mailings;
 - Religious, political, or other research that does not fall within the ALGSA's areas of interest, as described in Section I of these Research Grant Proposal Guidelines;
 - Miscellaneous expenses unrelated to the research project including but not limited to journal subscriptions, advertisements, tuition fees, professional society dues, meals, receptions, or parking fees
- B. Research grant funds awarded by the ALGSA must be used for the specific purpose for which they were originally intended in the grant proposal. Any funds not used in the manner specified in the grant award must be returned to the ALGSA. Applicant(s) may apply for an extension of time to use remaining funds at the end of the grant period.
- C. Grant recipients shall provide comprehensive Research Grant Progress Reports (due dates below) summarizing results obtained with funds provided by the ALGSA. A template for the Progress Report is included as the last two pages of these Guidelines. The Report must indicate plans for publication of research findings in scientific or medical journals. The ALGSA requests that all publications or presentations resulting from research funded by the ALGSA include an acknowledgement of this support and, where possible, our contact information. The Grant Recipient must also provide a lay summary of work to the extent appropriate for use within the Alagille Syndrome community.

IV. Proposal Instructions

No special forms are required. The full proposal and accompanying documentation must be submitted through ProposalCentral on the Altum platform at <https://proposalcentral.com/GrantOpportunities.asp?GMID=245>. Only proposals written in the English language will be considered. Incomplete proposals will not be considered.

A. **Format:** Proposals should be formatted according to the following guidelines on ProposalCentral:

- Abstract that addresses the following:
 - Why is the work important to those affected by ALGS?
 - How will the research project be accomplished?
 - Why is funding from the ALGSA essential to the project?
 - What are the long-term goals of the project?
- Required attachments:
 - Ethics committee approval
 - PI NIH format biosketch
 - Research and design methods
 - Research proposal
 - Specific aims

B. **Content:** Full proposals should include the following information. **The eight (8) page limit is a maximum and should not be exceeded including references.**

- **Title of research project:** Choose a title that is descriptive and specific, not general.
- **Applicant(s):** Provide name(s), degrees and relevant titles.
- **Contact information:** Provide Applicant(s) mailing address (including institution, department, pharmaceutical company, etc.), telephone number, and email address.
- **Grant amount requested:** Indicate the total dollar amount (\$US) requested from the ALGSA for the research project with a maximum of \$150,000 USD.
- **Abstract:** Provide an abstract that addresses the following: What problem does the research project address? Why is the work important to those affected by ALGS? How will the research project be accomplished? Why is funding from the ALGSA essential to the project? What are the long-term goals of this project?
- **Specific aims:** Describe concisely the specific goals of the research, including any hypotheses to be tested, and the project's objectives. **(One [1] page maximum)**
- **Research and design methods:** Describe the research design and methodology that will be used to accomplish the project's specific aims. Include the means by which data will be collected, analyzed, and interpreted. Describe any new methodology and its advantages over existing techniques. Discuss the potential difficulties and limitations of the proposed procedures and alternative approaches to achieve the project's aims. Provide a tentative sequence or timetable for the project. Describe facilities, laboratory space, and major equipment that are pertinent to the project. **(One [1] page maximum)**
- **Budget and justification:** Provide a detailed budget for the project with justification.
- **Other funding sources:** Indicate the amount and granting organization for any other sources of funding for the proposed research project. For the Applicant(s), provide a list of current funding support as well as awards received in the past two (2) years.
- **Project personnel:** Provide the name, title, and role of any individual who will be involved in the project, including the Applicant(s). Indicate the percent effort that each person is expected to devote to the project. Include NIH format biosketch for each project personnel and collaborators.

- **Organization support:** Provide a letter of organization (institutional or industry) endorsement of the research project, signed by an appropriate official and the organization's fiscal officer. Provide contact information for each.
 - **Human subjects and informed consent:** Regulations require that all affiliated institutions establish and maintain appropriate policies and procedures for the protection of human subjects. If applicable, briefly describe the population of subjects involved in the research project, the process for informed consent, and the means by which protection will be ensured. Proof of current or pending project approval by an Institutional Review Board (IRB) or similar oversight committee, and a copy of Patient Informed Consent approved by an IRB or similar oversight committee will be required prior to award if the proposal is selected for funding. If the research project is a multi-site study, informed consent document and IRB approval will be required from each study site.
 - **Animal studies:** All proposals must conform to regulations for the safe and humane treatment of animals. If applicable, briefly describe the animals to be studied, and measures to minimize pain and discomfort. Proof of current or pending project approval by an Animal Use and Protection Committee or similar oversight group will be required prior to award if the proposal is selected for funding.
 - **Ethics Committee approval:** Attach a copy of IRB Ethics Committee approval.
 - **Human gene therapy:** If the research project is a clinical study that involves human gene therapy, include a copy of the National Institutes of Health (NIH) Recombinant DNA Advisory Committee (RAC) review (or similar review if international Applicant), or waiver of review.
- C. **Submission:** Proposals and accompanying documentation should be submitted on ProposalCentral on the Altum platform at <https://proposalcentral.com/GrantOpportunities.asp?GMID=245>
- D. . Further information on research can be obtained from the ALGSA via the website (www.alagille.org) or by contacting alagille@alagille.org or (901) 286-8869.
- E. **Timeline:** Full proposals must be received by the ALGSA no later than 5:00pm US Eastern Time April 30, 2022. The selected proposal will be funded by the ALGSA on June 30, 2022. Funding is limited to \$150,000 USD payable over two years, \$75,000 on June 30, 2022, and \$75,000 on June 30, 2023. Year 2 funding paid out only after receipt of Progress Reports - due dates below. Funds will be awarded in US dollars.
- F. **Acknowledgement:** ALGSA will acknowledge proposals electronically within seven (7) days of receipt. Proposals will be reviewed for completeness within fifteen (15) days of receipt, and forwarded to the Grant Proposal Review (GPR) Committee. **Incomplete proposals will not be considered.**

V. Proposal Review

Grant proposals will be reviewed by members of the ALGSA Medical Advisory Board (MAB). Ad hoc reviewers will be used when appropriate. The MAB will review each proposal and present its recommendations to the ALGSA Board of Directors whose decisions on awards are final. Notifications of funding (or lack thereof) will be made approximately two (2) months after the proposal submission deadline.

The following points will be taken into consideration when proposals are reviewed:

A. Relevancy:

- Is the proposed research relevant to the research grant policy of the ALGSA (as outlined in Section I of these Research Grant Proposal Guidelines)?
- Does the research project address the ALGSA's funding priorities (as outlined in Section I of these Research Grant Proposal Guidelines)? If yes, which ones? If no, why not?

B. Significance:

- Does the proposal have scientific merit?
- Does the project address an important problem or critical barrier to progress in ALGS research?
- If the aims of the project are achieved, how will scientific knowledge, technical capability, and/or clinical practice be improved?
- How will successful completion of the project aims change the concepts, methods, technologies, treatments, services, or preventative interventions that drive ALGS research?
- Will the proposed research impact ALGS patient treatment in the short- or long-term?
- Will the proposed research lead to further extramural funding that will advance knowledge of ALGS?

C. Applicant(s)/Principal Investigator(s) Qualifications:

- Is the Applicant(s) qualified to carry out the proposed study?
- If early stage or new investigator(s), or in the early stages of independent careers, does Applicant(s) have appropriate experience and training?
- If established, has Applicant(s) demonstrated an ongoing record of accomplishments that have advanced their field(s)?
- If the project is collaborative, do the investigators have complementary and integrated expertise; are their leadership approach, governance and organizational structure appropriate for the research project?

D. Innovation:

- Is the proposal innovative with a high probability of success serving as a foundation to establish a novel line of research?

E. Approach:

- Are the overall strategy, methodology, and analyses well-reasoned, appropriate and feasible to accomplish the specific aims of the research project with potential problems, alternative strategies, and benchmarks for success presented?
 - Include a summary of project strengths and weaknesses.
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F. Environment:

- Will the scientific environment in which the work will be done contribute to the probability of success including institutional or industry support, equipment and other physical resources available to the investigators?

G. Responsiveness:

- Is the proposal clear and concise with appropriate literature cited and budget appropriate and adequately justified?

The Alagille Syndrome Alliance (ALGSA) appreciates the efforts of the scientific community and pharmaceutical industry to better diagnose, manage and treat Alagille Syndrome (ALGS). Each day, we enlist the expertise of professionals from many fields in a collaborative effort toward advancing recognition and understanding of this complex and rare condition. We are grateful for your interest in our mission, and our community greatly appreciates your submission of this research proposal.

A template for the Research Grant Progress Report is
Provided on the following pages.

Alagille Syndrome Alliance
Collaborative Scientific Research Grant Progress Report
1st Mid-Year Report Due - December 30, 2022
Year End Report Due - June 30, 2023
2nd Mid-Year Report Due - December 30, 2023
Final Report Due - June 30, 2024

Submit one (1) copy of the completed Research Progress Reports electronically (as an email attachment) in portable document format (.pdf) to alagille@alagille.org with “2022 ALGSA Grant Cycle Progress Report” in the subject line. Photographs may be attached in the same email in separate files.

PROJECT TITLE:

GRANTEE’S NAME:

EMAIL ADDRESS:

ORGANIZATION:

REPORT DATE:

REPORTING PERIOD:

GRANT AMOUNT:

NARRATIVE REPORT: To be completed by the Grantee. Please include answers to these questions, preferably in two to three (2 to 3) pages total.

****Terms may be abbreviated, provided that the complete term is used initially.****

1. WHAT WERE THE ORIGINAL OBJECTIVES TO THIS STUDY?

2. WHICH OBJECTIVES HAVE BEEN ACCOMPLISHED?

3. PLEASE SUMMARIZE THESE ACCOMPLISHMENTS.

4. WHICH OBJECTIVES HAVE NOT BEEN MET?

5. DESCRIBE ANY PROBLEMS IN MEETING THESE OBJECTIVES.

6. ANY BUDGETARY QUESTIONS?

7. FUTURE PLANS FOR THIS PROJECT?

8. PUBLICATIONS OR SCIENTIFIC PRESENTATIONS RESULTING FROM THIS PROJECT.

9. Individuals and organizations who have helped the Alagille Syndrome Alliance (ALGSA) sponsor research projects may ask for updates on studies currently underway. PLEASE PROVIDE A STATEMENT WRITTEN FOR THE GENERAL PUBLIC SUMMARIZING THE HIGHLIGHTS OF THIS REPORT (i.e., a lay summary). *These comments should be suitable for publication on the ALGSA website, on ALGSA social media channels and/or in the ALGSA e-Newsletter.*

10. Please provide three to four (3 to 4) publication quality photographs depicting the work accomplished by the Grantee as a result of receiving ALGSA grant funds. The photographs could be of the Grantee and their collaborators, research being conducted in the lab, etc., and will be considered for use in ALGSA publications, including ALGSA e-Newsletter, social media channels and website. Please include identifying information and suggested captions. *These photographs should be sent in electronic (.jpg, .png, or .tif preferred) format by email to alagille@alagille.org at the same time as this report.*

FINANCIAL REPORT: To be completed by the Grantee and/or the Grantee Organization's Fiscal Officer. Amounts must be listed in \$USD.

GRANT AMOUNT	\$
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PERSONNEL COSTS

Grantee(s) (Project Leader[s])	\$
Professional and Administrative Staff	\$
Clerical	\$
Outside Consultants (Explain)	\$
Other (Explain)	\$

1. TOTAL PERSONNEL COSTS	\$
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PROGRAM COSTS

Equipment and Supplies	\$
Travel	\$
Patient Care	\$
Other (Explain)	\$

2. TOTAL PROGRAM COSTS

\$

ADMINISTRATIVE COSTS

Office (Supplies, Phone, Postage, Copies, Etc.)

\$

Printing and Publication

\$

Other (Explain)

\$

3. TOTAL ADMINISTRATIVE COSTS

\$

TOTAL PROJECT COSTS (1 + 2 + 3)

\$