2017 Application Guidelines

for

BIO-THERAPEUTICS IMPACT AWARD

In this packet you will find:

Alex's Lemonade Stand Foundation evolved from a young cancer patient's front yard lemonade stand to a national foundation for childhood cancer.

Bio-Therapeutics Impact Awards address the existing funding gap for investigator initiated biological clinical trials for childhood cancers. A successful proposal is expected to accelerate delivery of high impact therapeutics to children.

Alex's Lemonade Stand Foundation
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Contact:
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Overview

***Description*:**
The ALSF Bio-Therapeutics Impact award provides funding to support investigator-initiated clinical trials using biologic therapies, including but not limited to immunotherapy (including cell therapies), gene therapy and small molecules. This grant category was initiated to accelerate the development of clinical trials for promising biologic approaches to treat childhood cancers.

ALSF's *Travel for Care* program is available to patients enrolled in the clinical trial supported by this award. A medical representative must apply on behalf of the families who meet our criteria as listed in the Travel for Care guidelines. The online request form must specify the clinical trial is funded by ALSF's Bio-Therapeutics Impact Award (alexslemonade.org.webform/travel-fund-application).

This funding opportunity includes support for the following:

Track 1: Clinical trial support where all pre-clinical studies have been completed, an IND has been approved and funding is needed to conduct and complete the clinical trial.

Eligibility (Clinical Trial P.I.):

1. Has completed pre–clinical studies in childhood cancer(s) and holds an approved IND.
2. Demonstrates a track record of implementing novel biologic therapies.
3. Has experience with executing and completing clinical trials and a history of meeting projected accrual targets in a timely manner.
4. Must have a well-defined plan and time frame to complete trial enrollment.
5. Investigator may be working within a consortium.

Funding Allocation: Up to $1.5 million in total costs may be requested over three years (not to exceed $500,000 per year). Year two and three are contingent upon review and approval of progress report and conference call.

Track 2) Pre-clinical bridge funding is available for one year to perform IND enabling studies. Subsequent funding of the trial proposed in the application will be contingent upon first obtaining all necessary regulatory approvals.

Eligibility (Investigator/Co-Investigator)

1. The Principal Investigator (PI) will be responsible for the studies. If the subsequent trial will be run by another investigator, this co-investigator must be identified in the application and meet the eligibility criteria.
2. PI proposes pre–clinical studies for childhood cancer that are required to prepare for IND submission or the PI has submitted an IND and follow-up studies are required for approval.
3. Demonstrates a track record of implementing biologic therapies.
4. Has experience executing and completing clinical trials that meet projected accrual targets in a timely manner.
5. Must have a well-defined plan to complete IND submission to be approved within one year of the start of the grant.
6. Must have a well-defined plan and time frame to complete trial enrollment.
7. Investigator(s) may be working within a consortium.

Funding Allocation: Up to $1.5 million in total costs may be requested.

- Pre-clinical study completion: In year one up to $100,000 is available to perform IND enabling studies. Clinical Trial: Year two IND approved, up to $500,000 may be requested in the Progress Report to conduct the clinical trial. Year three may be funded up to $500,000. If a fourth year is justified to

complete the trial, up to $400,000 may be requested.

- IND Submitted & Approval Pending: In year one up to $100,000 is available, however expenditures must be justified. If IND is approved and clinical trial will commence prior to the end of Year 1, a re-budget for new expenses may be submitted, up to $400,000. Subsequent years will then be funded up to $500,000.
- If the proposed *trial* for the therapeutic agent approved in the application is not feasible at the end of the "bridge" year, ALSF and the Scientific Advisory Board may elect to terminate the grant. If a new alternative therapeutic agent is indicated, ALSF may recommend application to the next funding cycle for scientific review.

Restrictions:

- Industry sponsored clinical trials, in which the P.I. has not led the development of the preclinical data, are not eligible.
- Consortia are not eligible.
- Funds will be granted to non-profit institutions or organizations operating in the U.S.or Canada. Principal Investigators need not be U.S. citizens.
- Please contact ALSF if you have any questions about the appropriateness of your research ideas for this funding mechanism via Grants@AlexsLemonade.org

Budget: A detailed budget and justification is required using the downloadable Standard Budget form provided on the ALSF website (alexslemonade.org/grants/guidelines). Please adhere to the allocations noted above. Funds may not be used for indirect costs. Budget items may include:

- Personnel salary: ALSF adheres to the NIH salary cap for the grant year for the Principal Investigator(s)
- Clinical trial operations including per patient billing for research costs
- Correlative research laboratory studies
- Equipment: must justify why this is essential to the project (up to $250,000 over three years)
- Travel for research personnel: must justify travel costs for protocol management/oversight and/or presenting results at scientific meetings

Review Process:

- Proposals must fall within the scope of ALSF's mission for an application to be considered.
- Applications will be reviewed by a panel of experts in pediatric cancer drug development who also have expertise in biologic therapies according to the NIH recognized peer-review process. The review process will be overseen by ALSF's Scientific Advisory Board.

Reporting:

- Progress Reports & Conference Calls:
  - Annual funding after the first year is contingent upon 1) the review of progress reports which must demonstrate satisfactory progress toward goals and the approved plan for enrollment targets, and appropriate expenditures, 2) a conference call discussion of progress hosted by ALSF.
  - If IND was pending at time of the application, subsequent funding is contingent upon a letter of confirmation from your institution along with a copy of the IND approval from the FDA for the clinical trial. If the IND is pending beyond the first year of funding, ALSF requires consultation with the PI and the Scientific Advisory Board before further funding is released.
- A final report is required at the conclusion of funding.

Application

All applications must be submitted using ALSF's two part online submission process. Applicants must first complete the online form then upload the application in one PDF (max 10 MB).

Part I: Online Form

A. Contact Information
Applicant will be asked for basic contact information for themselves, their institution and other key personnel.

B. Project Overview and Layman's Summary
Applicants must fill in their project title, requested amount, type of childhood cancer(s) being studied as well as provide a 250 word summary of the research project in layman terms. (You will be asked to release this summary for use at ALSF's discretion should proposal be funded.) Text may be copied into these sections.

Part II: Application Outline

- All sections described below should be combined into one PDF (max 10 MB) and uploaded to the ALSF online form.
- All templates may be found on ALSF's website: ALSFgrants.org using the Information for Grant Applicants button. They are also accessible while filling out the online portion of the application by clicking Forms and Guidelines download button at the top right of the page.
- All pages of the application should be numbered; the name of the principal investigator should appear in the upper right-hand corner of each page.
- Please follow NIH formatting guidelines: Arial, Helvetica, Palatino Linotype, or Georgia fonts with a font size of 11 points or larger and a minimum of ½ inch margins.
- Please follow the order of the outline below, adhering to the maximum limits for each subsection indicated in parentheses.
- Please cite references by number (Vancouver citation style)

A. Cover Page (1 page)
Complete the document provided on the ALSF website referenced above.

B. Table of Contents (1 page)
Please provide a Table of Contents with page numbers for the corresponding sections.

C. Project Information

1. **Impact Statement (1/2 page)** How will this project impact pediatric cancer patients?

2. **Scientific Abstract (1/2 page)** Please include a summary of the research objectives and rationale.

3. **Budget/Justification (3 pages)** Use the 5 Year budget template found on the ALSF website. No indirect costs will be paid. The signature from an institutional representative on the cover page of the grant application specifically acknowledges and accepts this provision.

4. **Biographical Sketch(es)** Include the NIH short biographical sketch for the Principal Investigator, Co-Investigator and key personnel.

5. **Project Details**

i. Research Plan (6 pages) Describe the project's research goals and the infrastructure you are requesting to accomplish the goals. Provide rationale for the request. Please include:

- Background and Rationale
- Preliminary Data
- Pre-clinical Studies (if applicable)
- Experimental Design of Clinical Trial
  - Target enrollment for trial (per year)
  - Plan to complete enrollment, including strategy for recruitment
  - Staffing requirements to open/run trial
  - Cost analysis
  - IRB & IND approval plans OR IND confirmation if already approved
- Detailed Statistical Plan
- Potential Problems and Alternative Methods

ii. Environment (2 pages) Describe your institution and, if applicable, collaborating institutions. Please include:

- An explanation that documents your institution's infrastructure and how it will provide support to perform clinical trials using biologic therapies.
- Institution's track record conducting clinical/translational research for childhood cancer over the last five years and current infrastructure for clinical trials.
- Number of patients enrolled per year for the last five years on Phase I and II trials.
- Number of Phase I and number of Phase II trials open per year for the last five years.
- Number of newly diagnosed pediatric cancer patients per year at your institution.

iii. Long Range Plan (half page)
Describe the long term vision for this trial <u>after</u> the end of ALSF funding.

iv. Literature Cited
Please use Vancouver Format (numbered references).

v. Collaborators and/or Consultants
Include one letter from each collaborator and/or consultant confirming their roles in the project.

D. Appendix

- Include the most current copy of the proposed clinical trial documentation, e.g. full protocol, protocol draft, concept or letter of intent.
- Additional appendices should be included ***only*** if essential to the understanding of the application and are limited to one accepted but not yet published manuscript or two pages of additional information and figures.
- Excessive appendices will cause the application to be rejected administratively.

Important Dates

Application Due Date	June 30, 2017 (11:59 PM ET)
Award Notification	October 2017
Project Start Date	February 1, 2018
Track 1: 3 Year Grant Clinical Trial only	
Year 1 Progress Report	January 2, 2019
Year 2 Progress Report	January 2, 2020 (estimate expenses through report period end)
Year 3 Final Report	March 1, 2021 (estimate expenses through report period end)
Track 2: 4 Year Grant with Pre-clinical Bridge	
Year 1 Progress Report & Proof of IND	January 2, 2019
Year 2 Progress Report	January 2, 2020 (estimate expenses through report period end)
Year 3 Progress Report	January 4, 2021 (estimate expenses through report period end)
Year 4 Final Report	March 1, 2022 (actual expenses through end of grant)

Submitting Your Application

All requests must be submitted using ALSF's online application.

1. Go to ALSFgrants.org and click the "Information for Grant Applicants" button.

2. Under **Resources for Applicants**, **Forms** download the Cover Page and Budget – 5 Year Grant.

3. **To start** an online application, under "**Get Started/Start a New Application**" click the drop down and select the Bio-Therapeutics Impact Award application link.
   - Returning users- login with your email address and password;
   - New users- click New Applicant link to set up an account with email address and password.

4. **Complete** the online form with contact and project information
   - Upload application as one PDF (maximum of 10 MB) – see format guidelines on previous pages
   - Review & Submit or Save your application and return at a later time to continue working

5. **To continue** your saved application, go to ALSFgrants.org and click the "Information for Grant Applicants" button
   - Under "**Get Started/ Continue a saved application or manage applications**", click the orange LOGIN button.
   - Click the name of the application on the "In Progress" list
   - Complete the form and upload the PDF
   - Review & Submit will send the application to ALSF

6. After your application has been successfully submitted an *email confirmation will be sent to your user account login email address*. You will not be able to amend the application.

Please contact Kay Schaul at 610-649-3034 if you have any questions about your grant application and submission or email questions to Grants@AlexsLemonade.org