



Foundation for Angelman Syndrome Therapeutics (FAST)

Innovative Methods to Progress Angelman Syndrome Treatments Towards Clinical Trials IMPACT Awards

PURPOSE

FAST's grants and fellowship programs aim to encourage therapeutic translational research initiatives, focusing on potential treatments as well as research related to the development of biomarkers and outcome measures. These awards are designed to significantly accelerate the progress of these platforms towards benefiting individuals affected by Angelman syndrome (AS).

At the core of FAST's mission is a commitment to supporting individuals living with AS and their caregivers, with the ultimate goal of helping them realize their full potential and bring scientific and therapeutic advancements that help to translate to the human condition.

FAST is committed to bringing practical treatments for Angelman syndrome (AS) into current medical practice as quickly as possible. While our priority is on funding translational and clinical research, we also support high-risk/high-reward discovery projects. FAST's research program awards grants to the world's best scientists who are focused on discovering and developing treatments, technologies and ultimately a cure benefiting those with AS. The focus of FAST-funded research is in supporting projects that have the potential for immediate and high impact on the Angelman community; therefore, priority will be given to research-based translational grants or those showing high promise for translating basic biomedical knowledge into clinical application.

IMPACT Awards

A new program called the FAST (Innovative Methods to Progress Angelman Syndrome Treatments Towards Clinical Trials) IMPACT grant program has been launched to support large therapeutic translational research platform projects from pre-clinical to IND-enabling stage. The FAST IMPACT award is open to investigators proposing novel approaches or repurposing approved or other clinically safe therapies evaluated in other disease indications that could advance Angelman syndrome research. Pharmacological



and biologic interventions, disease modifying therapeutics, as well as gene altering and gene replacement therapies are prioritized.

FAST will strongly consider programs where clinical and pre-clinical approaches are built on strong scientific and biological rationale for targeting fundamental processes underlying the Angelman syndrome cause. FAST will also consider large program proposals that could greatly enhance the Angelman syndrome community's progress to help de-risk various therapeutic approaches which could expedite moving forward to IND enabling studies or clinical readiness. Our goal is to provide funds to pursue and validate innovative ideas, support the researcher as she/he conducts translational studies, up to IND-enabling studies. Please note that FAST prefers to work closely with study investigators to ensure a plan is designed and executed with the greatest chance of success as the goal of these grants is to financially support the needs of a program and de-risk these programs for clear therapeutic potential. Award amount for FAST IMPACT Grant is up to \$1,000,000 per year. Final budgets will be determined based on review of proposed work and based on the extent of FAST's role.

LETTER OF INTENT

Applicants are required to submit a Letter of Intent (LOI) to FAST at grants@cureangelman.org to determine the feasibility of a potential request prior to applying. LOI's must be submitted as one PDF file containing the following information:

1. A brief description of the project including objectives and rationale;
2. An estimate of proposed budget and duration of the project;
3. Preliminary data to support the project; and
4. A CV/Biosketch

The entire LOI (minus the CV) should be no more than 3 pages.

KEY DATES

All grant applications have a rolling deadline. Please contact:

grants@cureangelman.org for more information and provide a letter of intent if you are considering applying for a grant.

ELIGIBILITY

Institutions receiving grants must be recognized as nonprofit. In the US, this means institutions that do not operate to make a profit. Individuals should have training and experience at least equal to the Ph.D. or M.D. level. Awardees are required to pursue



their research and academia requirements on a full-time basis, or as specified by the sponsoring institution in accordance with its own policies.

DURATION OF SUPPORT

FAST-IMPACT Awards are allocated based on the proposed needs of the project. Expedited timelines are encouraged, which will be made in either one- or two-year allocations, pending on the scope of work. Either one or two year grants have a maximum recommended funding of \$1,000,000 per year. In exceptional circumstances where larger grant amounts are needed, individuals may submit an inquiry, along with a letter of intent, to grants@cureangelman.org. This letter should provide a justification for the increased funding requirement.

For one-year grants, recipients are required to furnish a financial report and bi-yearly progress report within six months of the award activation date, and a final report 60-days following the conclusion of the grant if no Year 2 funding is being requested. Quarterly updates at both 3 and 9 months will be provided via email, video, or audio call to share progress with FAST throughout the award period. Second year funding is non-competitive but is based on review of the first year's progress report. For grants longer than 1 year, a grant renewal application must be submitted at least 8 weeks before the subsequent year of funding is requested along with a progress and financial report.

ALLOWABLE COSTS

Potential allowable costs include:

- **Reagents and Materials:** These may include but are not limited to: gene chips, chemical libraries, acquisition of cell lines, acquisition of animal model lines.
- **Services:** These may include, but are not limited to: deep sequencing, proteomics, metabolomics, mass spectrometry, antibody production, and drug/chemical manufacturing.
- **Equipment:** FAST will consider the purchase of specialized pieces of equipment or software purchases for specific purchases for specific projects when necessary.
- **Personnel:** Including salary and fringe benefits. Additionally, FAST will fund postdoctoral and graduate students at a salary equivalent to the current NIH guidelines.
- **Indirect Costs:** FAST allows up to 5% indirect cost rate to the budget's modified total direct costs (less those costs specifically excluded) to be included in the



total cost of the project. FAST will not pay any indirects on tuition, equipment, software or software licenses. Example: modified total direct costs of \$100, 5% indirects would be \$5; total cost of project is \$105.

- **Travel Costs:** Each award will include a \$3,000 travel stipend to allow for the PI and one trainee, when applicable, to travel to the annual FAST scientific symposium, where they may be required to present their current research progress.

APPLICATION and SUBMISSION INFORMATION

Write the research plan: Applicants should download the grant application form and prepare a Research Plan that emphasizes the methods and utilizes the following outline format:

1. **Abstract:** A succinct description of the proposed work that includes the project's broad, long-term objectives and specific aims, and a description of the research design and methods.
2. **Lay summary:** This should include your research objectives and your preliminary work that can be easily interpreted by a lay person. The focus should be on the translational component of your research objectives. (1 page maximum). We are committed to educating the public about the importance of this research and this will be posted on the FAST website if the project is funded.
3. **Budget Justification:** One page budget with justification is required.
4. **Other Support:** Please list all active and pending support to ensure there is no scientific, budgetary, or commitment overlap.
5. **Facilities and Resources:** This section should include any organizational resources available to perform the proposed studies. Identify the facilities to be used (Laboratory, Animal, Computer, Office, Clinical and Other). If appropriate, indicate their capacities, pertinent capabilities, relative proximity and extent of availability to the project. Describe only those resources that are directly applicable to the proposed work.
6. **Budget:** Applicants must use the budget sheet provided in application materials. If the proposal is multi-year, Applicants must use separate budget sheets per year, fill in the start and end date and applicable amounts for each year of support separately, summing for total amount requested.
7. **Hypotheses and Specific Aims:** Include specific research questions, objectives and hypotheses. The focus of the applications should be aligned with the FAST mission.



8. **Background and Significance:** Provide rationale for the research with emphasis on what is needed and why this project would benefit a translational therapeutic research program for Angelman syndrome.
9. **Preliminary studies:** Include any preliminary data you have gathered which supports the specific research question(s).
10. **Research Design and Methods:** Include study design, methods, and expected results.
11. **Limitations and Potential Pitfalls:** Discuss potential difficulties and/or limitations in achieving aims and alternative approaches to achieving aims.
12. **Milestone Timeline:** A research timeline will be provided with milestone goals to be reached during the funding period. These milestones will measure progress made throughout the award period and help determine Year 2 funding if applicable.
13. **Biosketch:** The biosketch of the PI and any collaborative personnel for the project. Please use the current NIH Biosketch format.

FORMATTING

- Proposal in Arial or Times New Roman size 11 point font, single or 1.15 spacing;
- Margins no less than 0.5”;
- Include a header on each page indicating name of PI (Last, First), page number;
- Figures and figure legends included within the 5 page limit; and
- Only PDF files accepted for review.

SUBMIT APPLICATION MATERIALS

Applications must be submitted electronically to grants@cureangelman.org. Only PDF files will be accepted and reviewed. Applications will be accepted on a rolling basis. Any questions about the application process should be directed to grants@cureangelman.org.

SIMILAR or IDENTICAL APPLICATIONS

FAST will not consider grant applications with essentially the same research focus from the same applicant organization for the same submission date. This includes derivative or multiple applications that aim to create a single product, process, or service that can be applied to various purposes with minor modifications. Applicant organizations must confirm and ensure that the materials they submit on behalf of the principal investigator



are original and have not been utilized elsewhere in the preparation and submission of a similar grant application.

HUMAN and ANIMAL SUBJECTS

Human subjects studied in the course of research conducted under a research award are under no circumstances the responsibility of FAST. Human subjects in a program supported by FAST shall be volunteers in any survey, study, or procedure. FAST requires recipients of any award using human subjects to demonstrate institutional review board (IRB) approval of the research project prior to dispersal of the awarded funds. If IRB approval cannot be obtained within 6 months of the award, the award may be rescinded by the Board of Directors.

If animals are used in the proposed study, written certification must be provided to FAST indicating that proper treatment, care, and humane conditions will be provided. FAST requires recipients to demonstrate Institutional Animal Care and Use Committee (IACUC) approval of the research project prior to dispersal of award funds.

FAST GRANT REVIEW PROCESS

The first stage of providing fair and expert review for research funding applications submitted to FAST consists of scientific peer review by a group of highly esteemed basic and clinical scientists and clinicians that form our Scientific Advisory Board (SAB). The panel uses standard guidelines established by the National Institutes of Health (NIH) for scoring applications with an emphasis on innovation, scientific rigor, and relevance to the mission of FAST. All reviewer conflicts of interest must be revealed prior to the review. All members of the SAB have signed a nondisclosure/confidentiality agreement. If the SAB Chair deems there to be a conflict of interest, the SAB member in conflict will be recused from the review process. Conflicts of interest include, but are not limited to, employment at the same sponsoring institution and collaboration on recent or current research projects, competing research proposal for the same request for proposal (RFP), and anything else that the chair of the SAB determines to be a conflict.

Applicants undergo scientific peer-review by the SAB and will receive scores based on scientific merit, innovation, translational impact and alignment with FAST's mission. Applications will also be reviewed based on the experimental design, methods, rationale, budget and feasibility. Applicants should concisely and adequately detail how the hypotheses will be tested, demonstrating adequate power for testing the hypothesis,



and clearly define all variables and aims. If SAB critiques have follow-up questions or recommendations, Applicant will provide a response to each question and/or recommendation. The SAB recommendations are then reviewed and compiled by the Scientific Director Panel. This panel consists of the Chair of the SAB, the CSO and the Science Director. All recommendations for funding are made by the panel to the FAST Board of Directors (BOD). Panel funding recommendations are based on scientific merit (as evaluated by the SAB), budgetary considerations and discussion of relevancy and priority to the mission of FAST. Final approval is required by the FAST BOD before funding may occur.

FAST strives to review and give a funding response to all submitted grants within six weeks of receiving the grant submission. This allows for the grants to be initially reviewed and for any concerns raised by the SAB to be properly addressed by the Applicant before final funding decisions are made.

NO-COST EXTENSIONS

A no-cost extension extends the project period beyond the original project end date. No additional funding will be allowed. A no-cost extension may be requested if there is a programmatic need to continue the research and there are sufficient funds. A no-cost extension request should be made at least 30 days prior to the end of the project period and must be requested by the PI detailing the amount being requested, the cause for such request, and the date in which the extension would end.

CARRY-FORWARD POLICY

A carryover allows unobligated funds remaining at the end of the budget period to be carried forward to the next budget period. The carryover would allow the grantee to use the unused prior year funds in a following budget period. A carryover request should be made at least 30 days prior to the end of the project period and must be requested by the PI detailing the amount to be carried over, explanation of unobligated balance, detailed budget and scientific justification. A carryover request will only be allowed for grants longer than one year.

PROHIBITION OF DISCRIMINATION

No person shall be excluded from participation in or denied the benefits of any program or activity receiving financial assistance from FAST because of race, ethnic origin, religion, age, sex or sexual orientation.



AMENDMENT OF POLICIES

FAST reserves the right to modify its policies governing research awards at any time. The recipient agrees to abide by any changes or to terminate the grant at the time when such changes become effective. Failure to abide by the policies governing awards shall be considered sufficient grounds for cancellation of an award or refusal to consider any pending application by the grantee.