

Fragile X Research Funding of \$1 Million Offered for 2022



Grant Application Deadline: February 1, 2022

FRAXA plans to fund a generous number of research grants and fellowships this coming year. Our mission is to find specific treatments and ultimately a cure for Fragile X syndrome. We aim to bring practical treatment into current medical practice as quickly as possible; we prioritize projects that have a clear practical application and the results of which will be shared in a timely fashion.

FRAXA awards two categories of grants:

1. **Clinical Trial** applications are accepted at any time. Our #1 priority is to fund clinical trials, while still continuing to support worthwhile translational research and other early-stage work.
2. **FRAXA Fellowship** applications are due on February 1 each year, and new fellowships are announced in early April.

A brief Letter of Intent to [Michael Tranfaglia, MD](#) is essential for all applications.

- ▶ [Clinical Trial Grants](#)
- ▶ [FRAXA Fellowships](#)
- ▶ [Our Strategy](#)
- ▶ [Application Process](#)
- ▶ [Renewals](#)
- ▶ [Final Reports](#)
- ▶ [Indirect Costs](#)
- ▶ [Reporting and Publicity](#)
- ▶ [Dissemination of Results, and Intellectual Property](#)

Questions?

Contact Michael Tranfaglia with any questions, comments or concerns via email at mtranfaglia@fraxa.org or call (978) 462-1866.

Clinical Trial Grants are Top Priority

FRAXA Clinical Trial Grant applications may be submitted at any time, and the budget is flexible.

These grants are strictly for clinical research with human subjects who have Fragile X.

- Flexible in amount, structure, and duration, but may be subject to some negotiation.
- Emphasis will be placed on clinical trials of potentially disease-modifying agents which have been previously validated in Fragile X animal models. Other types of clinical research will also be considered, such as biomarker studies in Fragile X patients, or trials of symptomatic therapies.
- No limit to structure of grant (can fund PI, postdoc, grad student, technician, supplies, etc.) or time-frame (though all grants over one year still need yearly renewal)
- No limit on amount, but please remember that applications are ranked based on relative value, so smaller requests have an advantage!

FRAXA Fellowships offer \$90,000 over Two Years

FRAXA Fellowships provide \$45,000 per year for up to 2 years. Application deadline is midnight on February 1.

- Priority is given to research with a translational or preclinical focus on Fragile X, which has potential to lead to improved treatment.
- Fellowships are initially funded for one year and are renewable for a second year, assuming successful progress and timely submission of a [renewal application](#).
- Any university-based lab in the world may apply.
- Fellowships can provide salary support for one scientist (any degree of BS or higher) working at least ½ time on the project.
- Supply budget is allowed.
- Limit of one application per lab group, and current grantees are not eligible.
- Non-transferable (only good for specified postdoc, PI, and institution).
- PIs must demonstrate adequate overall funding to pursue the project. Successful applicants for these grants will likely be working in established labs which have funding for supplies and any required animal handling costs. If the applicant proposes to work with a particular Fragile X model (mouse, drosophila, neural stem cells, etc.) s/he should demonstrate that this model system is already established in-house.



Our Strategy

FRAXA Research Foundation focuses on research which is likely to lead to new and improved treatments – and ultimately a cure – for Fragile X syndrome, with an emphasis on disease-modifying therapeutics based on understanding of Fragile X disease mechanisms. Every year we receive proposals from scientists worldwide seeking funding for the most cutting-edge Fragile X research. FRAXA has funded more than \$30 million in medical research.

Our goal has always been to accelerate the pace of research by eliminating “bottlenecks” in this long and complex process. Our funding priorities are determined by the state of the research and the nature of these bottlenecks, so we constantly re-examine these priorities as the field progresses.

In FRAXA's early years, little was known about basic disease mechanisms, so we placed great emphasis on funding basic research to understand the pathophysiology of Fragile X. Also, research in the Fragile X field was initially the province of molecular biologists and geneticists, so bringing more neuroscientists into the research community was deemed a high priority. These past efforts have been quite successful, generating detailed insights into the basic biology of Fragile X. Fragile X and the normal function of FMRP are both hot topics in neuroscience today. Thus, basic research into Fragile X disease mechanisms can no longer be considered a bottleneck and is now a lower priority for funding.

Other past bottlenecks, such as poor antibodies to FMRP and difficulties obtaining animal models, have been resolved by specific [resource](#) grants by FRAXA (some continuing to the present). This has enabled an explosion in translational research in Fragile X, leading to many promising therapeutic strategies in the works.

However, this also had the net effect of moving the bottleneck further down the road, to the preclinical validation stage of the development process. In response, FRAXA has developed new methods of validating promising drug treatment strategies in a standardized manner, with vastly greater efficiency. We are now less likely to fund academic labs for years on end to test one particular drug strategy – this approach, though appropriate in the past, is too inefficient for the task moving forward. With the bottleneck at the preclinical validation stage of development essentially resolved, the new bottleneck down the road is now clinical trials.

Application Process

Deadline for receipt of FRAXA Fellowship applications is midnight eastern time on February 1 each year. Clinical trial applications are accepted at any time. Email submission is required; please email your application as single pdf file to mtranfaglia@fraxa.org and kclapp@fraxa.org. Please be sure we acknowledge receipt of application. Applicants will be notified of awards in early May. Successful applicants and their university grants offices will be required to sign FRAXA's [grants contract](#).

Please provide the following information with your application:

- Description of the proposed project (8-15 pages recommended).
- Lay description suitable for publishing on fraxa.org if project is funded.
- Curriculum Vitae for the Principal Investigator.
- Curriculum Vitae for the Fellow to be supported under the grant and any other team members on the project.
- 3 references who are willing to be contacted to provide recommendations for candidate fellow.
- Budget.
- Full accounting of any other current and submitted sources of support for this project and other lab research.
- Requested start date. The earliest possible start date is 3 months after receipt of the application.

Renewals

FRAXA Fellowships are made for one year and may be renewed for a second year based on satisfactory progress. For FRAXA grants of more than one year duration, we require a renewal application be submitted at least 8 weeks before the second year of funding is needed.

Final Reports

For every FRAXA-funded project, we require a final scientific report within three months following the award period. In addition, we require a non-confidential lay summary to be posted on fraxa.org.

[Final Report Guidelines](#)

Indirect Costs

FRAXA is unable to pay for indirect costs under any circumstances.

Reporting and Publicity

- Before a project can start, the scientists funded will work with FRAXA to develop an announcement of the project for fraxa.org and social media. The grantee institution, Principal Investigator, and Fellow will cooperate fully with FRAXA in announcements through the news media of grants awarded and in materials prepared to publicize work under the grant. FRAXA will publish periodic descriptions and updates on funded projects on fraxa.org.
- In accepting a grant, the funded scientists and applying institution undertakes that at its conclusion it will submit promptly to FRAXA a complete final report on the work pursued. On awards of more than one year duration, a yearly scientific report will be expected. In addition, institution will supply a yearly accounting of funds received from FRAXA and expenditures.
- Scientists will recognize the support of FRAXA in any published article resulting from the work pursued with a credit line reading "Supported in part by a grant from the FRAXA Research Foundation"
- Scientists will recognize the support of FRAXA in any presentation of the work at professional meetings and conferences.

Dissemination of Results, and Intellectual Property

FRAXA's purpose in providing grants is to accelerate progress towards treatment for Fragile X. Therefore, in accepting an award, the Principal Investigator undertakes to publish the results of the work pursued in the primary literature in a timely fashion. Once published, the reagents, methods, materials, and products of the work must be made available immediately to other qualified researchers."Invention" means all discoveries, improvements, inventions, new concepts and ideas arising from the study together with all related data, results and information. Title to any Invention shall reside with the grantee institution. The grantee institution will notify FRAXA promptly after identifying any Invention that the grantee institution believes is patentable, and will supply FRAXA with copies of that Invention. For a patented Invention conceived and reduced to practice that is subsequently licensed under this grant, FRAXA and the grantee institution will negotiate in good faith to enter into a licensing agreement consistent with the grantee institution's policies and procedures solely for the equitable distribution of net income from the invention, taking into consideration the level of funding provided.

Grantee institution shall make diligent efforts to license the invention for commercial development to a third-party. Unless otherwise agreed to by FRAXA, the institution or other title holder shall agree that, if it or its designee or licensee has not taken effective steps to bring the invention to practical or commercial application through licensing or otherwise on terms that are reasonable in the

circumstances within three years after issuance of the patent, FRAXA shall receive an automatic grant of a non-exclusive, indefinite, fully paid-up, royalty-free, transferable, irrevocable world-wide license (with the right to sub-licenses) to advance such invention within the field of Fragile X research, diagnosis, and treatment.

For further information please contact [Michael Tranfaglia](#)

Global Leader in Fragile X Research

FRAXA-funded researchers around the world are leading the way towards effective treatments and ultimately a cure.

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