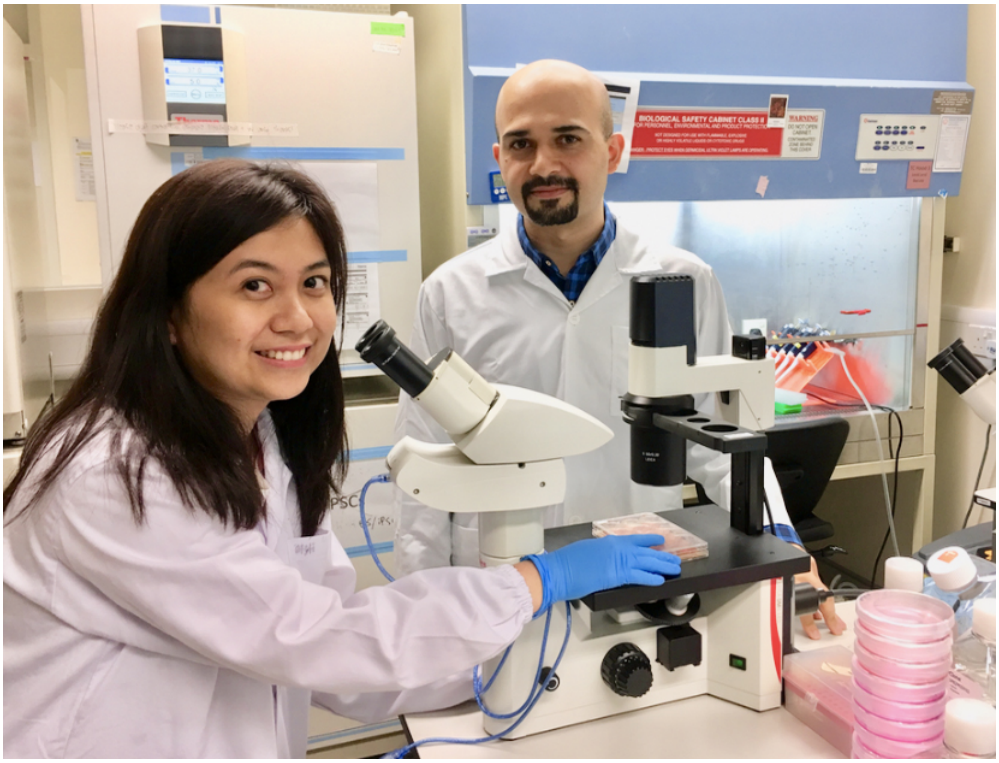
FRAXA Plans for Fragile X Research: 2019 Funding Opportunities

Michael Tranfaglia, MD FRAXA Updates • Research Updates

February 5, 2019

Fragile X Clinical Trial Proposals Welcome Anytime



2019 Focus is Clinical Trials

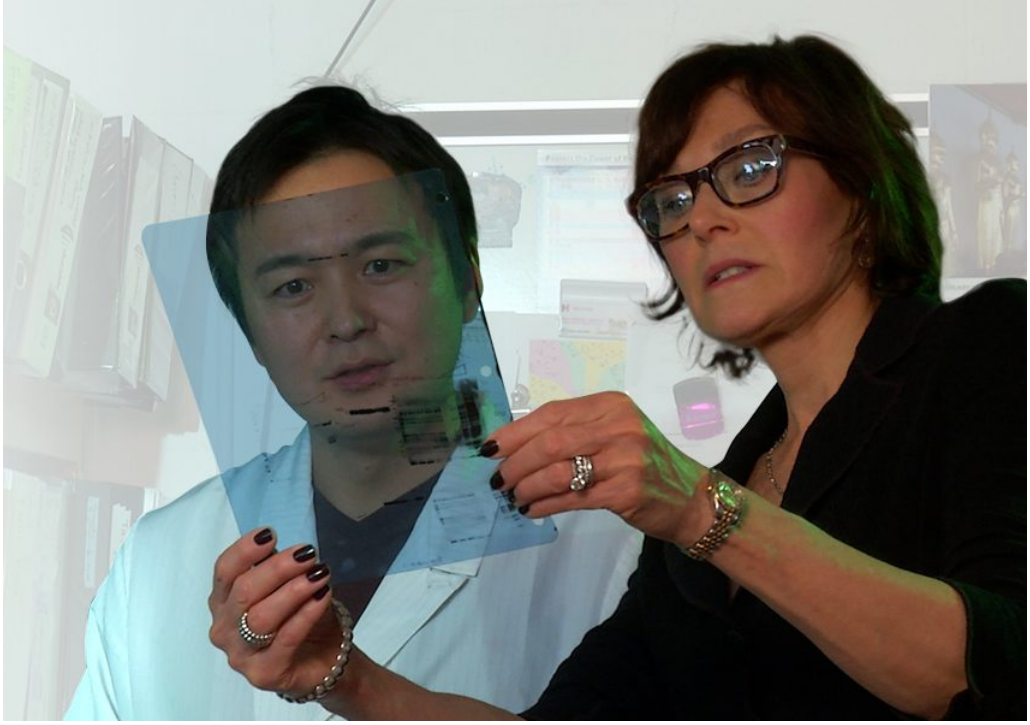
FRAXA awards two categories of grants, and guidelines for these grants have changed significantly from previous years. The purpose of the changes is to place a greater emphasis on clinical trials, while still continuing to fund worthwhile translational research and other early-stage work.

FRAXA Clinical Trial Grants: Applications may be submitted at any time. These grants are strictly for clinical research with Fragile X subjects. 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 may also be considered, such as biomarker studies in Fragile X patients, or trials of symptomatic therapies. Budgets and grant structure are flexible but may be subject to some negotiation.

The application deadline for **FRAXA Fellowships** is February 1 each year, and new fellowships are announced in early April.

A brief Letter of Intent to Michael Tranfaglia, MD is essential.

APPLY FOR GRANTS



Background and Rationale

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 $26 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 the early years of FRAXA's existence, little was known about basic disease mechanisms, so we placed great emphasis on funding of 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; additionally, Fragile X and the normal function of FMRP are both very hot topics in neuroscience today. Thus, basic research into Fragile X disease mechanisms can no longer be considered a bottleneck, and it must be considered a lower priority for funding.

Other past bottlenecks in research, 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 at the clinical trials stage.

Best of luck!

Michael Tranfaglia, MD
Medical Director, FRAXA Research Foundation

Explore Current Fragile X Research

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

CURRENT RESEARCH GRANTS

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