

Objective of the Discovery Grant

Accelerate Kabuki syndrome basic science research and the identification of 1+ new therapeutic opportunity (disease-causing target / pathway) through collaboration of a group of researchers from multiple institutions.

The Discovery Grant at a Glance

Type of research: basic science

Where you're based: worldwide

Level of funding: \$150,000 on average. Lower funding requirements that can achieve the objectives would be appreciated. KSF is also able to co-fund with other granting organizations, or add to funding from other organizations that would improve outcomes or accelerate timelines.

Duration of funding: up to 2 years; shorter grants that reach critical endpoints sooner are appreciated.

Who can apply

You can apply for this Grant if you want to work collaboratively, as part of a team, to accelerate basic science research and identify new therapeutic opportunities for Kabuki syndrome.

Each team member must be essential to the proposed collaborative research and have proven research experience in their field and in collaborative research. Team size will depend on the proposed research, but it has to be two or more people based at two or more institutions.

We encourage interdisciplinary research collaborations, although they are not essential.

Applicants will be considered from both the United States and internationally. Investigators from centers with a history of research in neurological / neurodevelopmental disorders are strongly encouraged to apply.

The average award is \$150,000; applications that achieve important outcomes for lower costs will be prioritized. Initial grants are awarded for up to two years with potential of renewal based on progress made during the two years.

KSF does not fund overhead for these types of grants.

Grant Application Guidelines

Applicants are invited to submit a full Grant application using the [application template](#) before the advertised deadline. Applicants should email the application in one electronic document (PDF format) to clara@kabukisyndrome.org with '*DISCOVERY GRANT: [Project title]*' in the subject.

Full applications should include the following:

- Project Title
- Project Team (Names, emails and institutions)
- Abstract (scientific summary of project) – no more than 350 words
- Lay summary of project – no more than 350 words
- Introduction
- Hypothesis to be tested
- Specific aims
- Significance of proposed research
- Methods and Procedures
 - Cells, animals, or patients selection/handling
 - The Grant may be used to cover the development of novel models or reagents required for the project, please justify this in the application
 - Research methods/tools
 - Materials used
 - Data collection methods and analyses - the appropriateness and rigor of the methodology and experimental design would be evaluated in depth. The applicant should set out and justify the following:
 - measures for avoidance of bias (for example, blinding, randomisation)
 - number of experimental and control groups and sample size per group
 - how the sample size was calculated, showing power calculations and including justification of effect size
 - overview of the planned statistical analyses in relation to the primary outcomes to be assessed
 - frequency of measurements and interventions to be used
 - circumstances in which power calculations are not appropriate to determine sample size.

- Anticipated results & potential pitfalls with some solutions
- Project timeline and expected milestones
 - Please segment your project into defined phases with clear milestones outlined
 - Continual payment of Grant will be linked to achieved milestones
- Project budget
 - Please break down the project budget into the phases defined in the project timeline. Each item and its cost must be clearly detailed in the budget and justified within the project description.
 - The award requires that funds not be used for the support of institutional indirect or overhead costs.

Letter of intent

Before submission of the full application, applicants are strongly encouraged to submit a letter of intent (LOI) using this [LOI template](#) to clara@kabukisynchromefoundation.org. The KSF review committee will review it and provide feedback within two weeks of the letter's submission. The last day to submit a LOI for feedback is 3 weeks before the advertised application deadline.

In the LOI, applicants should outline the following:

- Project Team (Names, emails and institutions)
- Summary of project with a brief outline of the methodology
- Aims of the research and anticipated results
- Significance of proposed research
- Estimate of timelines and costs

Although a letter of intent is not required, is not binding, and does not enter into the review of a subsequent application, the information that it contains allows KSF staff to estimate the potential review workload, plan the review and help potential applicants redirect the proposal if the planned project is out of scope.

Grant Review Process

1. Application submitted

Application round to open typically two months before the advertised deadline. Before submission of the full application, applicants are strongly encouraged to submit a **letter of intent** to the KSF research committee for feedback.

2. KSF research committee review

The KSF research committee reviews the Grant application to ensure it meets the Grant application criteria and identifies any gaps or additional questions for evaluation.

3. Submission of supplemental information if required

An opportunity for applicants to submit supplemental information and address the research committee's comments prior to review by subject-matter experts.

4. Reviewers evaluation

The Grant application (with any supplemental information) will be sent to a group of subject-matter experts bound by confidentiality, who will review the Grant application, provide feedback and score each application based on the evaluation criteria outlined below. Briefly, the evaluation will be based on several factors, including collaboration between institutions and researchers, the quality and feasibility of the Grant application, its alignment with the Grant's objectives, costs and timelines, and the applicants' capacity to carry out the project.

As much as possible, this process will be double-blinded, which means that neither the applicant nor the reviewer knows each other's identity.

After subject-matter experts have reviewed the application, the lay summary of top applications will also be reviewed by pre-selected members of the patient community for feedback to ensure applications resonate and meet the unmet needs of the community.

5. KSF Board decision

KSF Board of Directors make the final funding decision based on feedback from the subject-matter expert and community reviewers.

6. Funding decision announcement

Grant is awarded to the successful applicant and the grantee is then expected to carry out the proposed project within the terms and conditions of the Grant award.

2023 dates

- Application round opens: April 27
- Last day to submit a Letter of Intent for feedback: June 9
- Application deadline: June 30
- KSF research committee review: July 1-17
- Deadline for submission of supplemental information: August 11
- Expert reviewers evaluation: August 19 - September 18
- Community members evaluation: September 19-29
- Funding decision announcement: November

Application Evaluation Criteria

The application will be assessed according to the following criteria:

1. Significance, impact and relevance of the proposed research
 - How important are the research questions, or gaps in knowledge, that would be addressed?
 - What is the potential of the proposed research to advance the field?
 - How likely would the proposed research lead to significant new scientific understanding for Kabuki syndrome?
 - Consider the proposed research fit into the larger context of the field and its potential impact on scientific knowledge and implications for therapeutic development for Kabuki syndrome.
2. Methodology and feasibility of the proposed research
 - How convincing and coherent are the proposed research project methods and study design?
 - Are the proposed experiments and data analyses likely to achieve the proposed research objectives and milestones?
 - Is the approach innovative? Could it lead to new insights?
 - Have the project risks been well identified and will they be mitigated?
3. Collaborative plan

- Is the research team suitable to conduct the proposed research?
 - Does the team have the necessary collective expertise, skills, experience, capacity and resources to deliver the proposed research within the planned duration?
 - Is there a collaborative plan in place? Are the roles and responsibilities of each team member clearly defined and proposed collaboration feasible?
4. Timeline, milestones and financial plan
- Are the timeline, milestones, budget and proposed expenses accurate, appropriate and feasible?
 - Do the timeline, milestones and budget align with the proposed research methods and objectives?
 - Do the timeline, milestones and budget appear reasonable and realistic?

Each reviewer will score each award criterion on a scale from 1 (best) to 9 (worst), similar to the NIH grant application scoring system.

Table 1: Relationship between the level of impact, scores, and descriptors.

Overall impact/ criterion strength	Score	Descriptor
High	1	Exceptional. The proposal successfully addresses all relevant aspects of the criterion with essentially no weaknesses.
	2	Outstanding. The proposal addresses all relevant aspects of the criterion extremely strongly with negligible weaknesses.
	3	Excellent. The proposal addresses all relevant aspects of the criterion strongly with minor weaknesses.
Medium	4	Very Good. The proposal addresses the criterion very well, but numerous minor weaknesses are present.
	5	Good. The proposal addresses the criterion well, but at least one moderate weakness is present.
	6	Satisfactory. The proposal addresses the criterion well, but some moderate weaknesses are present.
Low	7	Fair. The proposal broadly addresses the criterion, but there are at least one major weakness.

	8	Marginal. The criterion is inadequately addressed, there are few major weaknesses.
	9	Poor. Proposal fails to address the criterion with numerous major weaknesses or cannot be assessed due to missing or incomplete information.
<p>Definitions:</p> <p><u>Minor</u>: easily addressable weakness that does not substantially lessen the impact of the project.</p> <p><u>Moderate</u>: weakness that lessens the impact of the project.</p> <p><u>Major</u>: weakness that severely limits the impact of the project.</p>		

Ratings are in whole numbers only, KSF expects that scores of 1 or 9 to be used less frequently than the other scores. Five is considered an average score.

After subject-matter experts have reviewed the full application, the lay summary of top applications will be shared with pre-selected members of the community for feedback.

The averaged scores, feedback from subject-matter experts and community reviewers will be shared with the KSF Board of Directors for their decision.

Grant conditions

As a condition of accepting funds from KSF, applicants must agree to the following conditions:

- Acknowledge funding provided by KSF in any publications or presentations of the research
- Make published results available to KSF for distribution
- Be available to attend KSF conferences to present the findings upon reasonable notice and where expenses are covered by KSF or by the Grant
- Share quarterly updates on research progress and an annual financial update with KSF