

[Patient-Partnered Collaborations for Single-Cell Analysis of Rare Inflammatory Pediatric Disease](#)

[Patient-Partnered Collaborations for Rare Neurodegenerative Disease](#)

FREQUENTLY ASKED QUESTIONS

This document aims to address frequently asked questions about these Requests For Applications (RFA). Many items are addressed in the RFA materials linked above and we encourage you to read those materials carefully. Please contact sciencegrants@chanzuckerberg.com with additional questions.

Does the patient partner need to be an official patient organization? I am a patient/patient's family member who has been working with researchers, but our group does not have official status as a not-for-profit?

The patient organization collaborator must be a tax-exempt 501(c)(3) patient-led organization that represents patients, engages patients in key leadership roles (i.e. as founder, executive director, board member, key staff, etc.) is patient-centered in its programming, and that focuses on a rare disease/disorder. Patient organizations that are fiscally sponsored are eligible to apply.

Can non-U.S. research institutions and non-U.S. patient organizations apply?

Yes! As long as the research institution or patient organization is a registered non-profit that is equivalent to 501(c)(3) organizations in the United States (an equivalency determination will be completed prior to funding).

Can non-U.S. and U.S. institutions/organizations apply together?

Yes! However, if there are U.S. and non-U.S. research institutions applying together, one of the US-based research institutions should be the applicant organization because non-U.S. institutions are prohibited from issuing funds to U.S. institutions in this RFA. Since patient organization funds are awarded separately from research institutions, patient organizations can be based either in the U.S. or outside of the U.S. regardless of where the research institutions are located.

Can current CZI grantees apply?

Yes! Current CZI grantees are eligible to apply as long as all other eligibility criteria are met. The project must be a new project or new research direction that is not currently funded by either CZI or other funding sources.

Do I have to be a CZI Rare As One (RAO) Network member or a CZI Neurodegeneration Challenge Network (NDCN) member to apply?

No. This is not part of the eligibility criteria for either RFA.

Does the lead patient organization PI have to have an academic appointment?

No. Only the research institution PIs are required to have an academic appointment and be in an independent faculty position or equivalent at an accredited college, university, medical school, or other research facility at the time of grant start. Independence is demonstrated by institutional support for independent research activities.

Does the lead patient organization PI have to be employed at the patient organization?

The lead patient organization PI should be either a staff member of the patient organization or play a key role on the organization's board. While it is not required that the lead patient organization PI themselves be a patient or family member, the organization as a whole must be patient-led in that it represents patients, engages patients in key leadership roles, and is patient-centered in its programming.

Is the priority with these RFAs to fund diseases with limited scientific research or limited funding?

A key aim of these RFAs is to encourage research where research has been limited; rare disease areas where there are currently robust, mature research efforts and active research communities and/or significant funding by federal and philanthropic initiatives will be of lower priority. We acknowledge that "significant" is difficult to define in this case. For these RFAs, clear demonstration of "significant" funding would be patient organizations with budgets in excess of \$5M USD or a large concerted effort from a funding agency such as NIH.

How can I get advance feedback on my proposal?

We are handling all inquiries by email so that we can assure that all potential applicants receive the same information. We are happy to reply to general questions regarding the grant program via email. To be fair to all applicants, CZI does not offer feedback on proposals in advance of submission or advise on application strategy.

The disease we work on is not well characterized. There are some hints that it may be neurodegenerative but we don't yet know this definitively. Would initial characterization of a disease with potential neurodegenerative or inflammatory features be within scope for this RFA?

This RFA scope is limited to rare neurodegenerative and pediatric diseases with an emphasis on understanding underlying biology and mechanisms. We appreciate that for some rare diseases, the characterization of the disease may be very limited and it may not yet be clear whether the disease is neurodegenerative by clinical or pathophysiological criteria. In those cases, the characterization of the disease may be too premature to support a strong research proposal focused on a specific and robust scientific question, which likely will limit the success of the application in this particular RFA.

Is there a specific role for the clinician PI on these projects?

Clinicians are a natural link between patients, patient organizations, and basic scientist researchers. They are also present in the patient experience from diagnosis through delivery (and discovery) of treatments. As such, we believe that clinicians play a critical role in these projects. The full extent of their role may differ in various projects, but could range from being a primary research lead (including being the coordinating research-PI/lead research PI) to serving as an interface between the patient community and researchers, supporting the flow of ideas, supporting sample collection, supporting understanding of disease manifestation, and providing an end-view (as diagnosticians/providers of treatments) perspective to the project. While drug discovery/clinical trial work is out of scope, projects focused on disease mechanism or disease stratification will inform such future work, and having the input and participation of a clinician in the design and interpretation of these projects will be critical to helping ground the work in a way that it feeds into next steps in the disease area as efficiently and meaningfully as possible.

FOR NEURO RFA ONLY: The RFA description says “to advance the understanding of the pathophysiology and mechanistic underpinnings of rare neurodegenerative and neurological disorders” Is this RFA open to disease areas where it is not clear whether they are neurodegenerative, for example, neurodevelopmental, intellectual disabilities, or other neurological disorders, like autoimmune that affect the nervous system?

This RFA scope is limited to rare neurodegenerative diseases. Neurological, neurodevelopmental, and neuropsychiatric disorders that are not considered neurodegenerative by consensus clinical criteria are not within scope for this RFA. The application includes a section for a clinical description of the disease, including evidence that the disease is neurodegenerative.