

# Chan Zuckerberg Initiative

## REQUEST FOR APPLICATIONS Rare As One Network (Cycle 3)

The Chan Zuckerberg Initiative invites applications for five-year projects from patient-led rare disease advocacy organizations to join the Rare As One Network. Grantee organizations will accelerate research within individual disease areas and work across specified areas of scientific focus (channelopathies; ciliopathies; or inborn errors of metabolism) to develop shared research priorities and proposals.

Learn about our Rare As One grantees from [Cycle 1](#) and [Cycle 2](#), and view the [Cycle 1](#) and the [Cycle 2](#) RFA.

### OPPORTUNITY

#### Overview

At the Chan Zuckerberg Initiative (CZI), we believe in supporting and leveraging the power of patients to accelerate research and drive progress against rare diseases. That's why — with continued guidance from patient communities, rare disease experts, and advocacy organizations — we are continuing to lift up the work of patient communities. Through the [Rare As One Project](#), we will scale a model in which patients, researchers, clinicians, and others work together to advance progress against their diseases.

Through the Rare As One Network (Cycle 3) Request for Applications (RFA), **CZI seeks to create a cohort of patient-led rare disease advocacy organizations that are dedicated to accelerating research across three classes of disease: channelopathies, ciliopathies, and inborn errors of metabolism (IEMs)**. CZI is inviting patient-led rare disease advocacy organizations focused on diseases within these three scientific focus areas to apply for a five-year grant opportunity to join the Rare As One Network. CZI will provide awardees with support to accelerate research within their individual disease areas and to work across their area of scientific focus (channelopathies; ciliopathies; or inborn errors of metabolism) to develop shared research priorities and proposals.

CZI will provide support for up to 30 patient-led organizations to strengthen organizational and scientific capacities; assemble or strengthen a collaborative research network, including basic researchers focused on the area of scientific focus; convene their disease community; and develop a patient-prioritized research agenda within the disease area. Throughout the grant period, CZI will provide grantee organizations with capacity building support, including funding, training, community learning opportunities, and mentorship. In turn, we ask the organizations to

participate as active members of the Cycle 3 cohort and overall Rare As One Network. This includes participation in regular calls, convenings, and collaborating with the other organizations within their area of scientific focus to identify shared research priorities and to develop research proposal(s) that could be eligible for funding under a possible supplemental CZI grant program.

## ELIGIBILITY

- Applicant organizations must:
  - Be patient-led advocacy groups, disease foundations or organizations that represent patients, employ patients in key leadership roles (e.g., Founder, Executive Director, Board of Directors), are patient-centered in their programming, and are representative and inclusive of the community they serve.
  - Be dedicated to accelerating research toward treatments and cures for a community of patients (i.e., not solely pursuing an n-of-1 therapeutic approach)
  - Be freestanding and self-governed (i.e., not a chapter or regional affiliate of a larger organization, not an international chapter of a nonprofit based in another country).
  - Be focused on a rare disease, disorder, or syndrome, or group of closely related rare diseases, disorders, or syndromes (either as defined in the U.S., as a condition that affects fewer than 200,000 people, *or* based on the relevant definition in the country or region where the organization is based).
  - Represent a disease or diseases that fall into one or more of the following three areas: (i) Channelopathies, a heterogeneous group of disorders resulting from the dysfunction of ion channels located in the membranes of all cells and many cellular organelles; (ii) Ciliopathies, a group of genetic disorders caused by structural or functional disruption of cilia, or by abnormal cilia biogenesis, including both motile and immotile/primary ciliary disorders; and/or (iii) Inborn Errors of Metabolism (IEMs), a group of diseases that result primarily from defects in specific enzymes that lead to the failure of the metabolic pathways involved in the breakdown or storage of various substances, such as carbohydrates, fatty acids, and amino acids.
  - Be tax-exempt under section 501(c)(3) of the Internal Revenue Code, have a valid fiscal sponsor that is tax-exempt under section 501(c)(3) of the Internal Revenue Code, or be a non-U.S.-based nonprofit/charitable patient-led rare disease organization that is equivalent to a 501(c)(3) designated organization. All grants will be awarded to organizations, not individuals.
  - Have an annual budget of less than \$5 million USD averaged over a two year period.
- Organizations that have been previously funded through any of the following multi-year CZI Science grant programs **are not eligible to apply**:
  - Rare As One Network Cycle 1
  - Rare As One Network Cycle 2 (*note: Cycle 2 applicants who received [one-time awards of \\$50,000](#) are eligible to apply*)
  - Patient-Partnered Collaborations for Single-Cell Analysis of Rare Inflammatory Pediatric Disease
  - Patient-Partnered Collaborations for Rare Neurodegenerative Disease
- Meta employees, including employees of any subsidiary Meta entities, as well as employees of Chan Zuckerberg Initiative, LLC, are not permitted to apply.

- CZI reserves the sole right to decide if an applicant organization meets the eligibility requirements.
- CZI reserves the right to request budget changes prior to award.
- We welcome applications from any country, provided the proposed work is compliant with the United States Treasury Department’s Office of Foreign Asset Control (OFAC) sanctions program. Prior to award, all grant applications will be reviewed for compliance with the United States Treasury Department’s Office of Foreign Asset Control (OFAC) sanctions program, the United States Department of Commerce’s export administration regulations, the Foreign Corrupt Practices Act (FCPA), any other applicable U.S. laws and regulations, and any corresponding laws and regulations in the country where the applicant is based. All grant agreements will require the grantee to comply with these laws and regulations. For additional information, please refer to: the [U.S. Treasury Department’s resources](#), the International Trade Administration’s [website on US Export Controls](#), and the Department of Justice’s [website on the FCPA](#).
- While applicants from all countries are welcome to apply, because of required ongoing compliance with U.S. sanctions and export controls, an applicant’s funding eligibility may need to be reassessed if the applicable laws and regulations change at any time. As a result, even if an applicant is eligible to receive funding at the time the application is reviewed, the applicant’s status may change later in the process or during the course of the grant term.

For questions about eligibility for this award or the application process, please contact us in advance of the proposal deadline at [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com). Deadline extensions will not be granted.

**Diversity and Equity:** We believe the strongest teams — encompassing ourselves, our grantees, and our partners — incorporate a wide range of backgrounds, lived experiences, perspectives, and voices that guide them to the most important unsolved problems. To enable our work, we incorporate diverse perspectives into our strategy and processes, and we also seek to empower community partners to engage in science. We are committed to building a grant program that fosters diversity and addresses systemic inequities. We are working to build tools and infrastructure and disseminate methods and skills, aiming for conditions in which a diverse range of prospective patient advocates can thrive and grow. Women, those with disabilities, underrepresented racial and ethnic groups, and organizations representing disease areas that disproportionately impact underrepresented or underserved communities are strongly encouraged to apply.

## APPLICATION REQUIREMENTS

### Key Dates

November 30, 2023:	Application portal opens
February 22, 2024:	Applications due by 5 PM (Pacific Time)
Early August 2024:	Earliest notification of decisions (subject to change)
October 1, 2024:	Expected start date of award period (subject to change)

**Award period and start date:** Awards will be five years in duration with an expected start date of October 1, 2024.

**Budget:** Awards will be \$800,000 total costs over five years (inclusive of up to 15% indirect costs). Indirect costs cannot exceed 15% of direct costs.

Funds may be used to support the development and maintenance of a research network for the organization's disease area of focus, convene the patient and research community, and align the community along shared research priorities. The funds may also be used to support staff and general operations. The funds may not be used to directly fund research, although they may be used to develop research-enabling infrastructure (e.g. biobanks, data collection efforts).

A high-level budget will be required in the application. The Chan Zuckerberg Initiative will work closely with finalists to arrive at a mutually acceptable budget after review.

All applications must be completed and submitted through CZI's online grants management portal at <https://apply.chanzuckerberg.com>. It is recommended that applicants familiarize themselves with this portal well in advance of the application deadline. Detailed application instructions are available on the [Chan Zuckerberg Initiative website](#), as well as in the [grants management portal](#). We also invite you to watch a short training video, [The Basics of Grant Writing](#).

## SELECTION PROCESS

The Chan Zuckerberg Initiative's [core values](#) center around people, technology, collaboration, and open science. We adhere to those values in both proposal selection and evaluation of progress. CZI will evaluate all applications and will seek independent expert review. CZI reserves the sole right to not recommend the funding of any applications. CZI does not provide feedback on decisions for unfunded proposals.

### Prioritization Criteria

While the application will be reviewed in full, and multiple factors will be evaluated, applications will be prioritized in the review process based on the following criteria:

- **The governance and community representation of the organization**, as demonstrated by degree to which the organization is patient-centered and representative of the population it serves, as reflected in both the diversity and capacity of the organization's leadership (including staff, board, etc.) and in its activities (including current or planned efforts to reach and serve patients, families, and communities of color affected by the disease).
- **The capacity of the leadership team to lead the development and expansion of a patient-led research network and other key organizational efforts**, as demonstrated by the motivation, commitment, experiences, and ability of the Patient Organization Lead and other key decision-makers within the organization.
- **The potential of the organization to benefit** from funding, organizational and scientific capacity building support, and the opportunity to participate in the Rare As One Network, as demonstrated by the organization's strengths, needs, and collaborative orientation.

- **The current state of the research community**, and how patient-led collaborative research could create a more inclusive community and accelerate progress in the disease area.
- **The need for and potential impact of expanded patient leadership and meaningful incorporation of the patient voice** to advance scientific progress in the disease area, and the capacity of the patient organization to serve in this role.

## POLICIES

- Funds from this award are intended to support rare disease organizations in the development of patient-led research networks. Grants are made to organizations and reasonable flexibility on how these funds are utilized is allowed, provided that the funds are used to support the development and maintenance of a research network for the organization's disease area of focus, convene the patient and research community, and align the community along shared research priorities.
- For awarded projects, financial statements and progress reports will be due at the conclusion of each grant year and occasionally more frequently. Specific deliverable requirements will be outlined in the award notification. Grantees of funded projects will be asked to participate in regular virtual trainings and monthly networking calls, with an estimated time commitment of 4-5 hours per month, in addition to in-person events which typically take place annually. Travel support for any in-person meetings will be provided by CZI separately from the requested grant funds.
- Grantees may obtain funds for their research from other funding sources, provided that there is no conflict with meeting the terms of the CZI award.
- Unused grant funds may be carried over to the following year, and requests for no-cost extensions will be considered at the end of the overall project period and upon receipt of an annual report.
- Indirect costs cannot exceed 15 percent of direct costs. Indirect costs may not be assessed on capital equipment or subcontracts, but subcontractors may include up to 15 percent of indirect costs of their direct costs.
- International grantees must use all grant funds exclusively for activities conducted outside the United States of America. Travel expenses to the United States must not be covered by the requested grant funds.
- **Ethical Conduct:** CZI advocates the highest standards for the ethical conduct of research. In addition to requirements of their own countries, grantees must adopt procedures for the use of animals in research and for the ethical treatment of human subjects and tissue donors, including obtaining their or their appropriate proxy's written informed consent. CZI regards the policies of the National Institutes of Health as a strong model for such procedures.
- **Data, publication and dissemination:** To accelerate scientific discovery and collaboration, CZI supports a consent, sharing, and publication policy for open and rapid dissemination of research results, including methods, data and reagents, and a policy for software development that maximizes accessibility, reuse, and shared development. Under rare circumstances, exceptions to the above may be considered where there are specific situations that make meeting these goals impossible or counterproductive to the project.
  - **Software Code:** CZI requires sharing of software code developed by its grantees generally to be made publicly available on GitHub (or a similar public service). All new code must be released under a permissive open source license (MIT, BSD

2-Clause, BSD 3-Clause, or Apache v2.0). All pre-existing and derivative code must be licensed under the most permissive license possible, given the licensing terms of the pre-existing code. All analysis packages must be released through the appropriate language-specific package manager (e.g., PyPi for Python, Bioconductor and CRAN for R) with documentation, example data, and interactive demos (e.g., Jupyter notebooks), and the use of Docker or similar container technologies to ensure portability and reproducibility. Software code supported by CZI must be archived for [long-term digital preservation](#) and [citability](#), when applicable.

- **Content and Data Sharing:** CZI is committed to developing and using platforms that disseminate data openly and freely. Any datasets either curated or generated through the proposal must be made as publicly available and easily accessible through an appropriate [data repository](#) as legally permissible, when applicable, under an [Open Definition conformant license](#). Ideally data sets would not include personally identifiable information, but if they do, consent to sharing the data must be obtained. Metadata, documentation, and intended use cases, as appropriate, must be made available under an Open Definition conformant license, preferably CC0 or CC BY/CC BY SA for content that requires explicit attribution.
- **Publications:** To encourage rapid dissemination of results, any publications related to this funded work must be submitted to a preprint server (such as bioRxiv, medRxiv, arXiv, or any appropriate preprint repository), at or before the first submission to a journal. Experimental protocols should be made publicly available through a protocol sharing service, such as protocols.io. CZI requests that scientific publications, preprints, and presentations that result from this award acknowledge support from this funding.
- **Reagent Sharing:** Resources and reagents developed with this funding support must be available for rapid dissemination to the community, where possible in an accessible community repository, such as Addgene (for plasmids/DNA reagents/viruses), Jackson Labs (for model systems lines), etc. This requirement applies to cell lines, transgenic organisms, plasmids/clones, antibodies, and other reagents.
- **Consent:** All human tissues must be adequately and fully consented to permit maximal sharing of the resulting data and any resulting tools, subject to applicable laws, regulations, or institutional ethical requirements. Any desired exceptions to this policy must be identified at the time of application, and such requests may affect the application's chance of success. We are aware that there may be circumstances where broad consent may be challenging, and in some cases consent may be subject to alteration or revocation; we encourage investigators to discuss these cases with CZI scientific staff.
- **Intellectual Property Rights:** CZI does not require assignment of ownership to any data, published results, or any other intellectual property that results from the work funded by these grants but will have the same rights generally granted to others. CZI supports and promotes policies that enable results and technologies to have the broadest reach and impact. To this end, all newly developed software must be made available through permissive open source licenses as described more fully above. Other technology and intellectual property rights (such as patents) must be made freely available for all academic and non-commercial use,

and where intellectual property rights are commercialized, they must generally be subject to non-exclusive commercial licenses that enable broad availability and dissemination.

- Applications selected through this process will either be funded by the Chan Zuckerberg Initiative Foundation (CZIF) or recommended for funding through the Chan Zuckerberg Initiative Donor-Advised Fund (CZI DAF) at the Silicon Valley Community Foundation (SVCF).

## **CONFIDENTIALITY**

All submitted applications will be kept confidential, except (1) as necessary for our evaluation or to comply with any applicable laws; and (2) to the extent that the application is made public or available to others without a duty of confidentiality through no fault of CZI. Notwithstanding, successfully funded proposals may be made publicly available and/or shared with other grantees or collaborators. Unfunded proposals will remain confidential as provided herein; however, information, including brief summaries of the proposed projects, project metrics, and the types of organizations that have applied for funding, may be made publicly available in aggregate form. Application materials will not be returned to applicants.

## **RFA CONTACT**

For administrative and programmatic inquiries, or other questions pertaining to this RFA, please contact [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com).

## **IMPORTANT DOCUMENTS**

[Application Instructions](#)



# Chan Zuckerberg Initiative

## APPLICATION INSTRUCTIONS Rare As One Network (Cycle 3)

Some helpful information as you get started:

- This document contains:
  - [General guidance on using the portal](#)
  - [How to submit an application](#)
  - [Application details specific to the Rare As One Network \(Cycle 3\) RFA](#)
- Please review the [Request for Applications](#).
- The Chan Zuckerberg Initiative (CZI) uses SurveyMonkey Apply (SMAppl) as its grants management portal. All applications must be submitted through this portal (<https://apply.chanzuckerberg.com>). SMAppl is configured to work best using the Google Chrome browser. It is recommended that you familiarize yourself with this portal well in advance of any deadlines. Deadline extensions will not be granted.
- **Key dates:**

November 30, 2023:	Application portal opens
February 22, 2024:	Applications due by 5 PM (Pacific Time)
Early August 2024:	Earliest notification of decisions (subject to change)
October 1, 2024:	Expected start date of award period (subject to change)
- **About this opportunity:** Through the Rare As One Network (Cycle 3) Request for Applications (RFA), CZI seeks to create a cohort of patient-led rare disease organizations that are dedicated to accelerating research across three classes of disease: channelopathies, ciliopathies, and inborn errors of metabolism (IEMs). CZI is inviting patient-led rare disease advocacy organizations focused on diseases within these three scientific focus areas to apply for a five-year grant opportunity to join the Rare As One Network. CZI will provide awardees with support to accelerate research within their individual disease areas and to work across their sub-cohort (channelopathies; ciliopathies; or inborn errors of metabolism) to develop shared research priorities and proposals.



CZI will provide support for up to 30 patient-led organizations to strengthen organizational and scientific capacities; assemble or strengthen a collaborative research network, including basic researchers focused on the area of scientific focus; convene their disease community; and develop a patient-prioritized research agenda within the disease area. Throughout the grant period, CZI will provide grantee organizations with capacity building support, including funding, training, community learning opportunities, and mentorship. In turn, we ask the organizations to participate as active members of the Cycle 3 cohort and overall Rare As One Network, including through participation in regular calls and convenings and collaboration with the other organizations within their area of scientific focus to identify shared research priorities and to develop research proposal(s) that could be eligible for funding under a possible supplemental CZI grant program.

- **Application specifics:**
  - **Eligibility:** Please refer to the [RFA announcement](#).
  - **Award Period:** Proposals should be five years in duration with an expected start date of October 1, 2024.
  - **Budget:** \$800,000 total costs over five years (inclusive of up to 15% indirect), distributed on the following schedule: Year 1: \$100,000; Years 2-4: \$200,000/year; Year 5: \$100,000. Indirect costs cannot exceed 15% of direct costs.

## GETTING STARTED

**Account setup:** The applicant (i.e., the person completing the application on behalf of the applicant organization) must first set up an account in the CZI online grants portal at <https://apply.chanzuckerberg.com/>.

To set up an account:

1. Go to <https://apply.chanzuckerberg.com/>.
2. Click the green **Register** button in the upper right corner.
3. Complete the requested fields and then click the green **Create Account** button.
4. Click the green **Continue** button to proceed to the site.

Please note you will need to verify your account through the auto-email that you receive after registering. You will not be able to submit an application until your account is verified.

**Personal data:** Where we ask for personal data of individuals in grant applications, please only submit personal data that you have a right to provide. We will use and store any personal data collected through the application process for grant-related purposes (e.g., administering the grant, analyzing and improving our grant practices). The Chan Zuckerberg Initiative Foundation and Chan Zuckerberg, LLC (collectively "CZI") will be the "data controllers" for any such personal information, and the data may be stored on servers outside of your home country, including within

the United States. If you have any questions or concerns regarding our privacy practices or collection or use of personal data, you can contact us at [privacy@chanzuckerberg.com](mailto:privacy@chanzuckerberg.com).

**Navigating the portal:** Once you have set up an account, you can log in to the grants portal at <https://apply.chanzuckerberg.com/>. Using the links in the upper right corner, you can access available programs (which includes RFAs for all CZI areas, not just Science) and any applications you have in preparation or previously submitted. Use the information (“i”) link to get help with the portal. To access your account information, click on your name in the upper right. Your application will pre-populate with the name and email listed in your account information so if you need to edit it, click on your name in the upper right corner to make any necessary changes.

**Forgotten username or password:** Please note that your username is your email address. If you have forgotten your password, please navigate to the grants portal at <https://apply.chanzuckerberg.com/> and click on the Log In button. Click the Forgot your password link and then enter the email address associated with your SMAApply account. You will then receive an email with information to reset your password.

**Other questions:** If you have other questions about using the portal, please use the information (“i”) link in the upper right corner of the window. Here you will find a link to FAQs about using the portal, as well as links to submit specific help requests. If you have specific questions about the RFA, please contact us at [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com).

## SUBMITTING AN APPLICATION

### To submit an application:

1. Go to <https://apply.chanzuckerberg.com/>.
2. Log in.
3. Click the green View Programs button that is displayed or click on the Programs link in the upper right corner. This will bring you to a listing of all programs/RFAs that CZI is hosting in SMAApply. To **find the program/RFA** you are looking for, you may need to scroll down.
4. Find the program/RFA you are interested in and click the green More button.
5. Click the green Apply button in the upper right and complete all sections (details below).
  - a. You will first be prompted to **enter the title** of your application (**which should be the name of your organization**), after which you will have access to the application tasks to complete. Project title is limited to 75 characters, including spaces. If you need to edit your project title, click on the My Applications link in upper right and click the green Continue button on the application you wish to edit. Once the application page opens, click on the three dots to the right of the application title (next to the Preview link) and select Rename from the dropdown menu.
  - b. The application is made up of several sections called **tasks** that are listed in a menu on the left side of the page. **For this RFA, the eligibility task must be completed before the other tasks will become available and visible.** It is strongly

- recommended that you complete the eligibility task/questionnaire well in advance of the submission deadline. Once the eligibility task has been completed, you can edit and complete tasks in any order. To **open a task**, click on the one you would like to work on in the table on the left hand side. You may need to scroll down to see the remaining tasks.
- c. Once you **complete a task**, click the green Mark as Complete button within the task. To **navigate to the next task**, click on the one you would like to work on in the table on the left hand side. All required tasks must be marked as complete before submitting. To **edit a task** after marking it as complete, click the three dots in the upper right of the task and select edit, which will re-open the task.
  - d. Your application will autosave every few seconds, but you can also click the Save & Continue Editing on each task as you go along to **save your application**.
  - e. In the tasks that require a PDF upload: If you need to **delete and replace a PDF** after you have uploaded it, click on the three dots to the right of the file under the Attach File section within the task and select Remove from the dropdown menu.
  - f. To **download your application**, click on the three dots in the upper right corner of the application page and select download. If you are within a task, first click on the Back to application link in the upper left. Please **be aware of any pop-up blockers** in your browser that may prevent downloading your application.
  - g. To **access an application that you have previously saved**, click on the My Applications link in upper right and click the green Continue button on the application you wish to edit.
6. Once all tasks are completed, click the green Submit button to **submit your application**.
    - a. If the button is grayed out, it means your application is not yet complete; please be sure all required fields and uploads are complete within each task and that you have clicked the Mark as Complete button within each task.
    - b. Review your application in the window (or in the PDF that you have downloaded- see 5f). If you want to make changes, navigate back to your application and reopen/edit any tasks that need editing. **It is strongly recommended that you download your application as a PDF (instructions above in 5f) to review your application before clicking submit.**
    - c. Once you are **ready to submit**, click the green Submit Your Application button on the left side of the window. You will need to confirm your submission by clicking the green Submit button in the pop up window. **Once your application has been submitted, it cannot be edited. Please be sure that your application is complete BEFORE submitting.** If you inadvertently submit your application and it is before the deadline, please contact [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com).
  7. Once your application is submitted, you will **receive an auto-email** confirming submission within a few minutes. If you do not receive a confirmation email within a few minutes, please check your spam folder. If you still did not receive your confirmation email, please email [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com).
  8. If you would like to view your application after you have submitted it, it can be accessed through the My Applications link in the upper right corner.

9. The grant writing process can feel overwhelming, especially for newer organizations. If you have not written a grant before, or would like to review tips from an experienced grant writer, we invite you to watch a short training video, [The Basics of Grant Writing](#).

**The application should be completed and submitted by the Patient Organization Lead. The Applicant/Patient Organization Lead must be affiliated with the organization listed, and grant funds will be awarded to this organization.**

**The application consists of the following sections (called tasks in the grants portal):** Eligibility, Patient Organization Lead, Equal Opportunity/Diversity (optional), Patient-Led Organization, Disease Area Details, Short Answer Questions

- **Eligibility:** In an effort to ensure your organization meets the eligibility requirements to apply, this eligibility questionnaire must be completed first. Once you have completed the questionnaire and confirmed your eligibility, the rest of the application will become available. **It is strongly recommended that you complete the eligibility questionnaire well in advance of the submission deadline.** No extensions will be granted.
  - Is your organization a patient-led organization (an advocacy group/disease foundation or organization that represents patients, employs patients in key leadership roles (e.g., Founder, Executive Director, Board of Directors)), patient-centered in its programming, and representative and inclusive of the community it serves?
  - Is your organization dedicated to accelerating research toward treatments and cures for a community of patients (i.e., not solely pursuing an n-of-1 therapeutic approach)?
  - Is your organization freestanding and self-governed (i.e., not a chapter or regional affiliate of a larger organization, not an international chapter of a nonprofit based in another country)?
  - Is your organization focused on a rare disease, disorder, or syndrome, or group of closely related rare diseases, disorders, or syndromes (either as defined in the U.S., as a condition that affects fewer than 200,000 people, *or* based on the relevant definition in the country or region where your organization is based)?
  - Does your organization represent a disease/diseases that fall into one or more of the following three areas:
    - (i) Channelopathies, a heterogeneous group of disorders resulting from the dysfunction of ion channels located in the membranes of all cells and many cellular organelles.
    - (ii) Ciliopathies, a group of genetic disorders caused by structural or functional disruption of cilia, or by abnormal cilia biogenesis, including both motile and immotile/primary ciliary disorders.

- (iii) Inborn Errors of Metabolism (IEMs), a group of diseases that result primarily from defects in specific enzymes that lead to the failure of the metabolic pathways involved in the breakdown or storage of various substances, such as carbohydrates, fatty acids, and amino acids.
- Is your organization tax-exempt under section 501(c)(3) of the Internal Revenue Code? Or, does your organization have a valid fiscal sponsor that is tax-exempt under section 501(c)(3) of the Internal Revenue Code? For non-U.S organizations, is your organization considered a nonprofit/charitable patient-led rare disease organization (equivalent to a U.S.-based 501(c)(3))?
- Does your organization have an annual budget of less than \$5 million USD averaged over a two-year period?
- Has your organization been funded through any of the following multi-year CZI Science grant programs?
  - Rare As One Network Cycle 1
  - Rare As One Network Cycle 2 (*note: Cycle 2 applicants who received one-time awards of \$50,000 are eligible to apply*)
  - Patient-Partnered Collaborations for Single-Cell Analysis of Rare Inflammatory Pediatric Disease
  - Patient-Partnered Collaborations for Rare Neurodegenerative Disease
- **Patient Organization Lead:** The information entered should be for the Patient Organization Lead (e.g. Executive Director, CEO, or head of the organization) who will be taking responsibility for the grant.
  - Name and email (auto-filled): To edit your name or email, click your name in the upper right corner and select My Account in the dropdown menu
  - Organization
  - Title/Position at organization: CEO/Executive Director, Board Member, Science Officer/Scientific Director, Other (please specify)
  - Paid/Unpaid
  - Connection to disease area: Patient/Parent of patient/Caregiver/Relative of an impacted patient/No personal connection to the disease area/Other (optional to specify)
  - Please upload a PDF of the Patient Organization Lead's resume.
  - If another member of your organization will serve as the administrative contact for the grant (including reviewing and responding to email from CZI, disseminating information to other members of the grantee organization, preparing and submitting grant reports, etc.) please provide their name, title and email address (optional)
- **Equal Opportunity & Diversity (optional):** CZ Science supports the science and technology that will make it possible to cure, prevent, or manage all diseases by the end of this century. Different communities are affected by or experience disease in different ways. Moreover, due to systemic barriers, the scientific enterprise itself is not a place

where all voices and talents thrive. We believe the strongest teams — encompassing ourselves, our grantees, and our partners — incorporate a wide range of backgrounds, lived experiences, and perspectives that guide them to the most important unsolved problems. To enable our work, we incorporate diverse perspectives into our strategy and processes, and we also seek to empower community partners to engage in science.

We request demographic information associated with applications submitted to CZI in response to our open calls. This information helps us learn from the RFA process, as well as improve our strategies to help ensure members of underrepresented or marginalized groups in science are aware of and able to apply to CZI opportunities.

**Please note that answering the questions below is voluntary, and receiving funding is not contingent on providing this information. Demographic information provided may be used in our grant-making process but will not be used as a factor in our grant funding decisions.** We may also publish aggregated data in various public forums, such as a website or blog. All responses will be shared only with limited personnel and service providers, who will use that information only for the purposes described in this paragraph.

If you have any additional questions about why we ask this, what we do with the data, or to share suggestions for improvement, please reach out to [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com).

The information may be entered for the intended Patient Organization Lead.

The categories listed below may not capture all possible identities; in the event that the categories do not accurately reflect your identities, please use the space provided to self describe.

- What is your race/ethnicity? (optional)
- What is your gender? (optional)
- Are you transgender? (optional)
- Are you a member of the LGBTQIA+ community? (optional)
- Do you have one or more disabilities? Please specify (optional)
- **Patient-Led Organization:**
  - Enter the organization's name/Street address/City/State/Country/Website/Year established.
  - Will the organization be applying with a fiscal sponsor? (Yes/No) If yes, provide:
    - Fiscal Sponsor Organization Name
    - Fiscal Sponsor Street address/City/State/Country/Website
    - Fiscal Sponsor Contact Information
      - First name, Last name, Title/Position, Email
  - Enter the organization's Employer Identification Number (EIN), as assigned by the Internal Revenue Service in the 9-digit format (XX-XXXXXXX; 10 characters

- total). If there will be a fiscally sponsored organization, please enter the EIN of the fiscal sponsor. Foreign organizations or others who do not have an EIN should enter 44-4444444.
- List the name and contact information for the person to discuss press releases and media.
    - First name, Last name, Title/Position, Email
  - Indicate the number of employees at the organization, including the Patient Organization Lead
  - Complete the table with the following information for each employee (up to a maximum of 10). **Please include the Patient Organization Lead and Administrative Contact (if different) in this section.** You may need to use the scroll bar at the bottom of the table to scroll right to view and complete all fields. Alternatively, you can tab to move through and complete the fields. For each employee, please enter:
    - Name, Title/Role, Full-time/Part-time, Paid/Unpaid, Years in Role
  - Share the organization's mission/mission statement (maximum of 75 words).
  - Mark all regions in which the organization provides support/engages in activities. Check all that apply.
    - Local, U.S., North America, South America, Africa, Asia, Europe, Australia
  - Describe which portions of the disease population the organization represents.
    - All, Pediatric only, Adult only, Other (please specify)
  - Are there other patient organizations working directly in the disease area your organization represents? (Yes/No)
    - If Yes:
      - List the names of other patient organizations directly involved in the disease area your organization represents.
      - Describe how your organization differentiates itself from and/or collaborates with these other organization(s) (i.e. in terms of mission/structure, involvement in research, services provided, etc.), how your organization contributes to or complements the work and efforts of other organizations, and how your organization ensures that its work is not duplicative (maximum of 150 words).
  - Has the organization previously received any funding or financial support from CZI? (Yes/No)
  - Organization Financial Information: provide the following information as a singular PDF upload. If the organization receiving the funds is a fiscal sponsor, please provide sections a. and b. for both the patient-led organization and the fiscal sponsor.
    - 1) Audited Financial Statements (if available): include the most recent fiscal year; if audited financials are not available, provide the unaudited version
    - 2) Organization Operating Budget - include:



- a. Current Fiscal Year Organization Budget (required); and
  - b. Forecasted Organization Budget which covers the duration of the potential grant (only if available)
- 3) Organization Size (required): provide the number of full-time employees employed by your organization, as of the current date
- 4) Project list of funders (required): High-level only, total funding, confirmed vs. unconfirmed for the specific project for all years of the grant
- Organization Budget:
  - Indicate what percentage of your budget is currently allocated to research (including direct research funding, development of research-enabling infrastructure, hosting scientific convenings, etc.).
- Project Budget:
  - Indicate how, if awarded, you would plan to leverage grant funds to fulfill the grant objectives (maximum of 150 words). (Note: per the grant requirements, funds should be used to support the development and maintenance of a research network for the organization's disease area of focus, convene the patient and research community, and align the community along shared research priorities. Funds may be used to support staff and general operations. Funds may not be used to directly fund research, although they may be used to develop research-enabling infrastructure (i.e. biobanks, data collection efforts, etc.)).
  - Upload in PDF format; provide a high-level, draft budget in tabular form, outlining \$800,000 total costs over five years (inclusive of up to 15% indirect), **distributed on the following schedule: Year 1: \$100,000; Years 2-4: \$200,000/year; Year 5: \$100,000** (one-page maximum).
    - Indirect costs are limited to up to 15 percent of direct costs. Indirect costs may not be assessed on capital equipment or subcontracts, but subcontractors may include up to 15 percent indirect costs of their direct costs.
    - Funds may be used to support the development and maintenance of a research network for the organization's disease area of focus, convene the patient and research community, and align the community along shared research priorities. The funds may also be used to support staff and general operations. Funds should not be used to directly fund research, although they may be used to develop research-enabling infrastructure (e.g. biobanks, data collection efforts).
    - Budget should be requested in U.S. dollars.
    - International grantees must use all grant funds exclusively for activities conducted outside the United States of America. Travel expenses to the United States (including round-trip tickets) should not be covered from the requested grant funds. Any attendance at

CZI meetings in the U.S. will be covered by CZI outside of requested grant funds.

● **Disease Area Details:**

- Provide the name of your organization’s rare disease, disorder, or syndrome, or group of closely related rare diseases, disorders, or syndromes of focus. Write out any acronyms. If the disease exists in a rare disease database such as [Gard](#), [Orphanet](#), or [Mondo](#), please provide the link to it (maximum of 3 rows).

For example:

Name	Link	Add another row ?
Charcot-Marie-Tooth disease type 2B	<a href="https://rarediseases.info.nih.gov/diseases/9192/charcot-marie-tooth-disease-type-2b">https://rarediseases.info.nih.gov/diseases/9192/charcot-marie-tooth-disease-type-2b</a>	X
Autosomal dominant Charcot-Marie-Tooth disease type 2B	<a href="https://bit.ly/2Yxqxgm">https://bit.ly/2Yxqxgm</a>	X
Charcot-Marie-Tooth disease type 2	<a href="https://monarchinitiative.org/disease/MONDO:0018993">https://monarchinitiative.org/disease/MONDO:0018993</a>	

- Select which one or more of the three CZI-specified areas of scientific focus (i.e., the sub-cohort(s) to which you are applying) is applicable to your organization’s disease area. Check all that apply.
  - (i) **Channelopathies**, a heterogeneous group of disorders resulting from the dysfunction of ion channels located in the membranes of all cells and many cellular organelles.
  - (ii) **Ciliopathies**, a group of genetic disorders caused by structural or functional disruption of cilia, or by abnormal cilia biogenesis, including both motile and immotile/primary ciliary disorders.
  - (iii) **Inborn Errors of Metabolism (IEMs)**, a group of diseases that result primarily from defects in specific enzymes that lead to the failure of the metabolic pathways involved in the breakdown or storage of various substances, such as carbohydrates, fatty acids, and amino acids.
- (Optional) if your disease is further sub-classified within the broader grouping of channelopathies, ciliopathies or IEMs, please describe (maximum of 30 words).
- Provide a published reference (link to a peer-reviewed paper or website) that associates your organization’s disease area with one or more of these areas of scientific focus for this RFA (channelopathies, ciliopathies, inborn errors of metabolism) or otherwise clearly demonstrates that the disease is one of these areas of scientific focus. Use URL format: <https://example.com>.

- (Optional) Are there specific diseases in the area(s) of scientific focus (i.e., ciliopathies, channelopathies, IEMs) that have similar cause, targets, or other commonality with your disease area that would benefit from a collaborative research approach? If so, please name them.
- Describe (a) the typical experience of patients with the disease, (b) the most common and prominent symptoms associated with the disease, (c) the typical progression of the disease, and (d) how the disease affects quality of life (maximum of 200 words).
- Indicate which organs and/or tissues are affected by the disease your organization focuses on. Check all that apply:
  - Bile ducts, Bladder, Blood, Blood vessels (veins, arteries), Bone marrow, Bones, Bowel, Brain, Breasts, Connective tissue / joints, Ductus deferens, Ears, Esophagus, Eyes, Fallopian tubes, Gallbladder, Heart, Intestines, Kidneys, Limbs, Liver, Lungs, [Lymph fluid, nodes, ducts, vessels], [Mouth / teeth], Muscles, Nerves, Nose, Ovaries, Pancreas, Parathyroid, Penis, Prostate gland, Scrotum, Skin, Spinal cord, Spleen, Stomach, Testes, Throat, Thyroid, Trachea, Uterus, Vagina, Vulva, Other (please specify), Unknown
- Indicate which body system(s) are affected by the disease your organization focuses on. Check all that apply:
  - Cardiovascular / Circulatory, Digestive, Endocrine, Hematopoietic / Lymphatic / Immune, Integumentary / Exocrine, Metabolic, Muscular / Skeletal, Nervous / Sensory, Renal / Urinary / Excretory, Reproductive, Respiratory, Other (please specify), Unknown
- Indicate the sex(es) of those affected by the disease. Check all that apply:
  - Male, Female, Intersex, Unknown
- Select the age group(s) affected by symptoms of the disease. Check all that apply:
  - Pre-birth, Infancy (Ages 0 to 1), Early childhood (1+ to 5), Middle childhood (6 to 11), Teen (12 to 17), Adult (18 to 65), Elderly (65+), Unknown
- What is the average age of diagnosis of an individual diagnosed with the disease? If this answer is dependent on sub-type, please explain in the provided text box (maximum of 250 characters).
  - Pre-birth, Infancy (Ages 0 to 1), Early childhood (1+ to 5), Middle childhood (6 to 11), Teen (12 to 17), Adult (18 to 65), Elderly (65+), Unknown, Depends on sub-type
- What is the average life expectancy (age) of an individual with the disease? If this answer is dependent on sub-type, please explain in the provided text box (maximum of 250 characters).
  - Pre-birth, Infancy (Ages 0 to 1), Early childhood (1+ to 5), Middle childhood (6 to 11), Teen (12 to 17), Adult (18 to 65), Elderly (65+), Unknown, Depends on sub-type

- Indicate, if known, the average length of time in years that passes between initial symptoms of the disease appearing and before a diagnosis is obtained. If unknown, please mark unknown.  
If this answer is dependent on sub-type, please explain in the provided text box (maximum of 250 characters).
    - Less than one year, 1 to 5 years, 5+ to 10 years, 10+ years, Unknown, Depends on sub-type
  - Indicate the typical method(s) of diagnosis of the disease. Check all that apply:
    - Clinical diagnosis, Neurological exam, Imaging, Genetic test, Blood test, Urine test, Biopsy, Metabolic test, Electrophysiological test, Other (please specify)
  - Indicate the prevalence of the disease (i.e. the approximate number of people believed to have the disease)
    - In the country where your organization is based
      - Less than 10, 11-50, 51-100, 101-1000, 1001-10000, 10,000+, Unknown
    - Beyond the country where your organization is based
      - Less than 10, 11-50, 51-100, 101-1000, 1001-10000, 10,000+, Unknown
  - Indicate the incidence of the disease (i.e. the approximate number of new cases identified per 100,000 people in a given year)
    - In the country where your organization is based
      - Less than 10, 11-50, 51-100, 101-1000, 1001-10000, 10,000+, Unknown
    - Beyond the country where your organization is based
      - Less than 10, 11-50, 51-100, 101-1000, 1001-10000, 10,000+, Unknown
  - Describe if there are global regions, ethnic, or racial groups in which there are higher than average prevalence/incidence rates (maximum of 75 words).
- **Short Answer Questions:**
    - **Organizational governance and community representation:** The degree to which the organization is patient-centered and representative of the population it serves, as reflected in both the diversity and capacity of the organization's leadership (including staff, board, etc.) and in its activities (including current or planned efforts to reach and serve patients, families, and communities of color affected by the disease).
      - Describe how your organization represents the interests of the patient community and is inclusive of and responsive to the patient community in its (a) governance and (b) outreach and other organizational activities (maximum of 150 words).
      - Describe what steps you are taking as an organization to improve diversity, inclusion, and belonging in your community, and to center and

- uplift equity for those who are underrepresented or marginalized in science or health care (maximum of 150 words).
- **Leadership Strength:** The capacity of the leadership team to lead the development and expansion of a patient-led research network and other key organizational efforts, as demonstrated by the motivation, commitment, experiences, and ability of the Patient Organization Lead and other key decision-makers within the organization:
    - Describe your leadership of the organization, including (maximum of 150 words):
      - Motivation for creating and/or leading this organization
      - Any previous experiences and/or skill set(s) that support your ability to lead the organization
      - Your leadership style and approach, including how you seek to inspire and engage others and support and enable them to achieve key goals
      - Your approach for developing and aligning around a strategic vision for your organization/disease area.
    - Describe your core leadership team (i.e. key leaders/decision makers (paid, unpaid, board members), including their (maximum of 150 words):
      - Experience and motivation
      - Skill sets
      - Identities and capacity to represent and engage the community, including those who are underrepresented or marginalized in your disease area.
  - **Potential to Benefit from Participation in the Rare As One Network:** The potential of the organization to benefit from funding, organizational and scientific capacity building support, and the opportunity to participate in the Rare As One Network, as demonstrated by the organization's strengths, needs, and collaborative orientation.
    - Describe what you see as your organization's greatest challenges and most pressing needs (maximum 150 words).
    - In addition to grant funds, CZI provides member organizations of the Rare As One Network with a variety of capacity building trainings and resources, to assist grantees in achieving the grant requirements and increase organizational sustainability throughout the funding period. These trainings and resources currently include monthly calls, small group discussions, 1:1 coaching with trainers and subject matter experts, mentorship with experienced rare disease advocates, office hours with CZI and external experts, in-person convenings and access to a community-specific portal. Grantees will be expected to commit an estimated 4-5 hours per month to virtual trainings and networking calls, in addition to in-person events, which typically take place annually. Please describe how the following Rare As One support would benefit your

organization and help address your identified challenges and needs (maximum 150 words):

- Grant funding
  - Participation in capacity building training and access to resources
  - Collaborating and engaging with other organizations in the Rare As One Network
- **Patient-Led Collaborative Research Network:** The current state of the research community, including how patient-led collaborative research could create a more inclusive community and accelerate progress in the disease area
- Describe the current state of the research community in your organization's disease area, and how you think the development or expansion of a patient-led collaborative research network would advance research and contribute to improved understanding of the disease (maximum of 150 words).
  - How might receipt of this grant enable you to develop or expand a collaborative research network in your disease areas? For example, are there specific fields, disciplines or areas of expertise that you would like to see reflected in your research network or that you think would bring important insights into the disease area (maximum of 150 words)?
  - Describe how the expansion of a patient-led collaborative research network in your own disease area – to include basic researchers focused on the area(s) of scientific focus you selected (i.e., channelopathies, ciliopathies and/or IEMs) – could make progress towards those priorities (maximum of 150 words).
  - Describe any challenges specifically related to representation or equity that affect research progress in your disease area and how expansion of the patient-led collaborative research network and/or engagement within the Rare As One Network could help to address them (maximum of 150 words).
- **Prioritizing and Pursuing Scientific Questions:** The need for and potential impact of expanded patient leadership and meaningful incorporation of the patient voice to advance scientific progress in the disease area, and the capacity of the patient organization to serve in this role
- Describe the role that your organization currently plays in the research community (maximum of 150 words).
  - Describe how your organization identifies the priorities and needs of patients (maximum of 150 words).
  - Describe the current state of the science in your disease area and existing research priorities (maximum of 150 words).
  - Describe how your disease area would benefit from the development of a patient-prioritized research agenda, and how your organization would engage the research network and patient community in that effort (maximum of 150 words).

- Describe how collaboration with other organizations within your area of scientific focus (i.e., sub-cohort—channelopathies, ciliopathies, IEMs) might contribute to understanding the fundamental biology of your disease (maximum of 150 words).
- What other possible benefits do you see arising from being a part of a sub-cohort of patient-led organizations (channelopathies, ciliopathies, IEMs)? How do you envision working with groups across the sub-cohort to identify common questions and research proposals? (maximum of 150 words).

The formatting and component requirements, including any word and/or page limits indicated above, will be enforced by the review team. Any submitted materials that exceed the word and page limits or do not follow the requirements will not be considered during the application review process.

## **QUESTIONS?**

For administrative and programmatic inquiries pertaining to this RFA, please contact [sciencegrants@chanzuckerberg.com](mailto:sciencegrants@chanzuckerberg.com).

For technical assistance with SMaply, please contact [support@smapply.io](mailto:support@smapply.io) or while logged into SMaply, click on the information "i" link in the upper right corner and submit a help request ticket.