CureAccelerator Live! Repurposing Research to Create Patient Impact in Rare Diseases and Diversity, Equity and Inclusion

Description / Background
Proposals are submitted on the Cures Within Reach CureAccelerator platform. Sign up as a Researcher or Clinician at https://app.cureaccelerator.org/registration or log into an existing CureAccelerator account at https://app.cureaccelerator.org/home to submit. Additional proposal submission instructions can be downloaded at this link: http://bit.ly/cwrcalrfp.

This Request for Proposals (RFP) from Cures Within Reach (CWR) is seeking clinical trials using repurposed treatments for any rare disease that falls within the Cures Within Reach US-focused Diversity, Equity and Inclusion efforts for an upcoming CureAccelerator Live! event. CureAccelerator Live! is a philanthropic pitch event, raising awareness of and funding for innovative repurposing research projects, with up to $50,000 total in funding for the winning project announced during the event. This RFP seeks clinical repurposing trials, up to five of which will be selected to pitch at this event, held on June 9, 2022 virtually and/or in Philadelphia, PA in conjunction with the Global Genes RARE Drug Development Symposium. Participation at this event may be in-person or virtual. More specifics about CureAccelerator Live! to come.

Eligible submissions must:

- Be interventional clinical trials using repurposed therapies that are already approved by the FDA, EMA or other regulatory agency or are otherwise readily available for human use
- Focus on an unsolved rare disease as defined by US standards as a condition that impacts fewer than 200,000 people or by European standards as a condition than impacts less than 1 in 2,000 people
- Be led by a US-based underrepresented racial / ethnic minority Principal Investigator (as defined by the NIH; Section A in https://grants.nih.gov/grants/guide/notice-files/NOT-OD-20-031.html) or address rare disease racial / ethnic health disparities in communities of color as defined by the NIH (see https://www.nimhd.nih.gov/about/overview), led by a Principal Investigator of any racial / ethnic background
- Be conducted at any US-based accredited academic, nonprofit and governmental research institutions and/or health systems significantly involved with medical research, where good scientific research and clinical practices can be assured

Rare disease clinical repurposing trials led by a US-based underrepresented racial / ethnic minority Principal Investigator may, but are not required to, focus on health disparities. CWR is interested in promoting Diversity, Equity and Inclusion in biomedical clinical research by 1) supporting the careers of and 2) closing the NIH funding gap for underrepresented racial /
ethnic groups through funding for clinical repurposing trials led by minority Principal Investigators.

CWR is also interested in supporting both previously funded investigators and early-stage investigators and who have received little or no extramural research funding to date. Principal Investigators who have not received extramural funding previously and/or who do not currently have their own lab should include a Letter of Support from a funded, senior researcher who will act as a mentor for the proposed clinical trial and the investigator.

Examples of rare disease racial / ethnic health disparities clinical repurposing trials include:

- A trial in a rare disease that disproportionately impacts communities of color, in incidence, prevalence or patient outcomes, and focuses enrollment on patients of color (examples: sickle cell disease, triple negative breast cancer, cystic fibrosis)
- A trial in a rare disease that includes specific enrollment goals to allow for data analysis by race / ethnicity
- A trial in a rare disease recruiting patients of color exclusively

Eligible clinical repurposing trials may also:

- Test combination therapies in order to increase efficacy, including combining the current disease treatment with a repurposed treatment or combining multiple repurposed therapies
- Modify current treatment protocols to make them more effective, helping more patients for longer periods of time
- Repurpose therapies approved for use in adults into pediatric indications, or vice versa

Up to five projects will be selected to be pitched at the CureAccelerator Live! event. Principal Investigators, or their designated and approved co-investigator, must be willing to make a pitch for their project either at an in-person event in Philadelphia, PA or at a virtual event on June 9, 2022. There are currently no travel stipends or reimbursements available. At least one pitched project will be selected for funding at the CureAccelerator Live! event.

All Principal Investigators selected as finalists for CureAccelerator Live! will receive consultation from Cures Within Reach for the preparation of their pitch, and Cures Within Reach will create a poster for the pitched project to be displayed during the event. Finalists will also provide a breakdown and justification for the total requested budget and a Research Roadmap that details the project’s research objectives, timeline and success measures.

Many of our successes in repurposing have come from researchers and clinicians who had ideas that could impact a disease that was outside their normal therapeutic or scientific area. Please submit your repurposing ideas, find colleagues to work with and circulate this RFP to others who you think might be able to submit repurposing research that could provide solutions for an unsolved disease.

While only clinical repurposing trials are eligible for this round of funding, we welcome proposals for any research stage / type, which may be considered for future funding opportunities. If you have a repurposing idea in a rare disease that isn’t an exact fit for this RFP, or if you have eligibility questions due to budget or other aspects, please contact Clare Thibodeaux, PhD at clare@cureswithinreach.org to discuss fit and/or submission options.

Important Funding Information:

Cures Within Reach will provide no more than 80% of the REQUESTED PROJECT FUNDING, and the institution or organization proposing the research will match at least 20% of the REQUESTED PROJECT FUNDING. The REQUESTED PROJECT FUNDING is
equal to the ESTIMATED OVERALL PROJECT COSTS in the BUDGET BREAKDOWN section of the proposal submission, and includes the 20% Institutional Match. The 20% Institutional Match must be real dollars committed to the project and can include Indirect Costs. The Institutional Match may come from the research institution itself (including salaries, patient costs, etc.); from government or other public funders; from individuals, foundations or other private funders; from industry (including the dollar value of donated drug); or from any combination of these sources.

All funding amounts and requests are in US dollars.

Funding Definitions:

- **Project Funding** = total research budget (no set maximum)
- **Remaining Project Funding** = funding needed to reach the total research budget; must be equal to the Requested Project Funding
- **Existing Project Funding** = Project Funding minus Remaining Project Funding (no set maximum)
- **Requested Project Funding** = funding requested through this RFP (maximum = $50,000, including the 20% institutional match)
- **Budget Breakdown** = breakdown of the Requested Project Funding (maximum total = $50,000, including 20% institutional match)
- **Estimated Overall Project Costs** = Requested Project Funding (see above)

Cures Within Reach will accept REQUESTED PROJECT FUNDING amounts that are within the minimum and maximum amounts indicated. We do not set an upper limit to the total PROJECT FUNDING, or the EXISTING PROJECT FUNDING, but REQUESTED PROJECT FUNDING amounts to Cures Within Reach are limited to the maximum amount specified in the Funding Information section below. Proposals may be submitted for which the PROJECT FUNDING is within the Cures Within Reach maximum, and for projects that already have EXISTING PROJECT FUNDING from another source and require REMAINING PROJECT FUNDING, when this additional funding from Cures Within Reach will help speed patient impact.

Cures Within Reach REQUESTED PROJECT FUNDING must be sole, late or final funding for the project. REQUESTED PROJECT FUNDING from Cures Within Reach cannot be the first funding raised for a project, unless the REQUESTED PROJECT FUNDING, including the 20% Institutional Match equals the PROJECT FUNDING.

Here are two examples of funding requests and disbursements to help investigators and institutions understand the funding opportunity.

**Example A**

The maximum funding request to CWR is $50,000. The PI enters $50,000 for REQUESTED PROJECT FUNDING and for ESTIMATED OVERALL PROJECT COSTS in the BUDGET BREAKDOWN, which equals the PROJECT FUNDING. CWR approves this project, and will provide $40,000 (80%) of the REQUESTED PROJECT FUNDING and the institution will match the remaining $10,000 (20%) of the REQUESTED PROJECT FUNDING.

**Example B**

The maximum funding request to CWR is $50,000. The PI enters $50,000 for REQUESTED PROJECT FUNDING and for ESTIMATED OVERALL PROJECT COSTS in the BUDGET BREAKDOWN, which will cover one-third of the $150,000 entered for PROJECT FUNDING. The other $100,000 is entered for EXISTING PROJECT FUNDING, and has already been secured by
the investigator and institution. CWR approves this project, and will provide $40,000 (80%) of the REQUESTED PROJECT FUNDING and the institution will match the remaining $10,000 (20%) of the REQUESTED PROJECT FUNDING and provide the $100,000 of the EXISTING PROJECT FUNDING.

Funding from Cures Within Reach cannot be used for any Indirect Costs, although the 20% Institutional Match may be used for Indirect Costs.

Diseases/Conditions

Any rare disease. A rare disease is defined by US standards as a condition that impacts fewer than 200,000 people, or by European standards as a condition than impacts less than 1 in 2,000 people.

Treatments

Any repurposed drugs, devices, nutraceuticals or diagnostics

Project Type

- Human Clinical Trial

Timeline

Do you have a preference for where the research should take place?

This research must take place in the United States. This RFP is open to US-based accredited academic and governmental research institutions and large group medical practices significantly involved with medical research. Although start-ups, biotechs, pharmaceutical companies and medical device companies are not eligible for this RFP, their collaborators at eligible institutions can apply. Contact Clare Thibodeaux, PhD at clare@cureswithinreach.org to learn more.

Do you have a preference for where you would like to see patient impact?

This RFP seeks clinical repurposing trials in any unsolved rare disease, that are:

- led by a US-based underrepresented racial / ethnic minority PI (as defined by the NIH; https://grants.nih.gov/grants/guide/notice-files/NOT-OD-20-031.html); or
- address rare disease racial / ethnic health disparities in communities of color as defined by the NIH (see https://www.nimhd.nih.gov/about/overview) led by a Principal Investigator of any racial / ethnic background

We are hopeful that this RFP can help address Diversity, Equity and Inclusion in rare diseases and that any discovery would be useful anywhere in the world where the repurposed therapy is available.

Restrictions
There are no other restrictions other than those described above.

**Other Information for Researchers**

If selected to participate in CureAccelerator Live!, the Principal Investigator will be required either to travel to Philadelphia, PA where the CureAccelerator Live! event will be held or to participate in a virtual CureAccelerator Live! event on June 9, 2022. There are currently no travel stipends or reimbursements available. More specifics about CureAccelerator Live! to come.

Cures Within Reach primarily funds proof of concept clinical repurposing trials that can determine whether a repurposed therapy can have a direct and positive impact on patients. Sometimes the clinical trials in our portfolio are open label studies with clinical endpoints that compare to the natural history of the disease. Other clinical trials are randomized, blinded and controlled studies. We are open to all clinical trial designs that have the opportunity to create a robust and well-defined outcome that will show reproducible clinical impact.

**Cost effectiveness, speed and patient impact are key ranking criteria for a successful proposal submission.** PIs will receive a decision on their proposal submission within approximately 4-6 weeks of the RFP deadline.

While only clinical repurposing trial projects are eligible for this round of funding, we welcome proposals for any research stage / type, which may be considered for future funding opportunities. If you have a repurposing idea in a rare disease that isn’t an exact fit for this RFP, or if you have eligibility questions due to budget or other aspects, please contact Clare Thibodeaux, PhD at clare@cureswithinreach.org to discuss fit and/or submission options.

**Funding Available**

Minimum $25,000

Maximum $50,000

**Funding Description**

See full Funding information in the Research Description section above. Cures Within Reach will accept REQUESTED PROJECT FUNDING amounts that are within the minimum and maximum amounts indicated. Cures Within Reach REQUESTED PROJECT FUNDING must be sole, late or final funding for the project, as indicated in the Research Description. Cures Within Reach will provide no more than 80% of the REQUESTED PROJECT FUNDING, and the institution or organization proposing the research will match at least 20% of the REQUESTED PROJECT FUNDING. The Institutional Match must be real dollars committed to the project and can include Indirect Costs. The Institutional Match can come from the research institution itself (including salaries, patient costs, etc.); from government or other public funders; from individuals, foundations or other private funders; from industry (including the dollar value of donated drug); or from any combination of these sources. All funding amounts are in US dollars.
Open to co-funding

Co-Funding Description

We will accept projects that already have funding from another source and require additional funding, when this additional funding from Cures Within Reach will help improve the chances of success of the project, and therefore increase the chance of patient impact. Cures Within Reach REQUESTED PROJECT FUNDING must be sole, late or final funding for the project, as indicated in the Research Description. We are open to working with other funders who share our desire to find solutions for rare disease patients and are interested in these near-term repurposing opportunities.

Due Date for Project Proposal Summary Submissions

03-09-2022