

CureAccelerator Live! Repurposing Research to Create Patient Impact in Rare Diseases

Description / Background

CureAccelerator! is a philanthropic pitch event, raising awareness of and funding for innovative repurposing research projects, with up to \$50,000 funding for the winning project announced that evening. This Request for Proposals seeks clinical repurposing trials, up to five of which will be selected to pitch at this live event, held in Spring/Summer 2019 in a US city. Exact date and location to be determined prior to the submission deadline.

This CureAccelerator Live! will showcase clinical repurposing trials in 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 that impacts less than 1 in 2,000 people. Eligible submissions can come from North America and Europe.

Repurposed therapies can be used alone or in combination with other therapies. **Therapies must already be FDA, EMA or any other regulatory agency approved or otherwise readily available for human use.** Additional Proposal submission instructions can be downloaded at this link: <https://goo.gl/MEuCWk>.

Our strongest preference is for proof of concept clinical repurposing trials supported by strong scientific evidence or clinical observations. We may also consider late stage preclinical projects that would lead directly to clinical repurposing trials. Late stage preclinical submissions would be strengthened by the support of a clinical team ready to begin a clinical trial, if the final preclinical work delivers the necessary data.

Cures Within Reach is circulating this RFP because our funders and scientists have determined that many rare diseases are either currently partially or fully unsolved. An unsolved disease is one in which one or more of the following are true:

- There is currently no effective treatment
- The current treatment is only effective for a portion of the patient population
- There is a treatment that is effective, but many patients develop resistance to the therapy
- There is a treatment that is effective for the entire patient population, but the treatment is very expensive, and therefore some patients cannot get access to the treatment
- There is a treatment that is effective for the entire patient population with significant side effects, and for some patients the negative side effects outweigh the benefits of the treatment

Up to five projects will be selected to be pitched at the CureAccelerator Live! finals event. Principal Investigators, or their designated and approved co-investigator, must be willing to attend the event and make a live pitch for their project to be selected. There are currently no travel stipends or reimbursements available. At least one pitched project will be selected for funding at the CureAccelerator Live! finals.

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.

Even if this is not your area of expertise, you may be able to help! 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. We are happy to receive proposals that would repurpose therapies from outside the rare disease area that could have an impact on rare diseases.

For example, a device that worked in eradicating liver cysts was repurposed by a team of researchers to create a repurposed treatment for prostate cancer. Gastroenterology and radiology researchers and clinicians worked with the oncology researchers and clinicians to assist in proposing and carrying out this repurposing research. So even if rare diseases aren't your area of expertise, you may still be able to help to make an impact on patients with rare diseases. **Please submit your repurposing ideas, find colleagues in this specialty to work with and circulate this RFP to others who you think might be able to submit repurposing research that could provide a solution for this disease.**

Important Funding Information:

Funding Definitions:

- Project Funding = total research budget (no set maximum)
- Remaining Project Funding = funding needed to reach the total research budget (no set maximum)
- 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 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% match from the institution, organization or company proposing the research, equals the PROJECT FUNDING.

Cures Within Reach will provide no more than 80% of the REQUESTED PROJECT FUNDING, and the institution, organization or company 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 institutional 20% match. All funding amounts and requests are in US dollars.

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

Example A

The minimum funding request to CWR is \$25,000 and the maximum is \$50,000. The PI enters \$40,000 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 \$32,000 (80% of the REQUESTED PROJECT FUNDING) and the investigator/institution will match the remaining \$8,000 of the REQUESTED PROJECT FUNDING.

Example B

The minimum funding request to CWR is \$25,000 and the maximum 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 investigator/institution will match the remaining \$10,000 of the REQUESTED PROJECT FUNDING.

Example C

The minimum funding request to CWR is \$25,000 and the maximum 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 investigator/institution will match the remaining \$10,000 of the REQUESTED PROJECT FUNDING and provide the \$100,000 of the EXISTING PROJECT FUNDING.

REQUESTED PROJECT FUNDING cannot be used for any indirect costs, although up to 10% of the REQUESTED PROJECT FUNDING can be used to cover direct project administrative 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 that impacts less than 1 in 2,000 people.

Treatments

Any repurposed drugs, devices, nutraceuticals or diagnostics

Project Type

Human Clinical Trial
Late Stage Animal Trial

Timeline

Project Must Start by: Fall 2019
Project Must Finish by: Within 36 months of start date

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

This research can take place in North America or Europe where good scientific research and clinical practices can be assured. This RFP is open to accredited

academic and governmental research institutions, large group medical practices significantly involved with medical research biotech, pharmaceutical companies and medical device companies.

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

Our main focus is on repurposing research in 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 that impacts less than 1 in 2,000 people. We are hopeful 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 to travel to the US city where the CureAccelerator Live! event will be held. The exact location will be determined prior to the submission deadline. There are currently no travel stipends or reimbursements available.

Cures Within Reach and our funders primarily fund 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.

While clinical repurposing trials are preferred, late preclinical studies may be considered. Any preclinical proposal submission would be strengthened by a supporting letter from a clinical collaborator willing to begin clinical trials once positive results are seen.

Cost effectiveness, speed and patient impact are key ranking criteria for a successful Proposal submission.

Funding Information

Minimum funding request to CWR \$25,000

Maximum funding request to CWR \$50,000

Funding Information:

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. REQUESTED PROJECT FUNDING cannot be used for any indirect costs, although up to 10% of the REQUESTED PROJECT FUNDING can be used to cover direct project administrative costs.

Co-Funding Description

Open to co-funding.

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

January 31, 2019