



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

## **Description / Background**

This Request for Proposals (RFP) from [Cures Within Reach](#) is seeking **clinical trials using repurposed treatments for any rare pediatric disease** 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 funding for the winning project announced that evening. This RFP seeks clinical repurposing trials, up to five of which will be selected to pitch at this live event, held virtually on June 10-11, 2021. More details about the event to come.

This CureAccelerator Live! will showcase clinical repurposing trials in pediatric rare diseases. 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 anywhere, with a preference for US-based institutions.

Repurposing research tests already approved and available therapies to determine if they are safe and effective in treating a different indication, thereby improving patient outcomes and quality of life. 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.** Repurposing research may also:

- Test combination therapies in order to increase efficacy, including combining the current disease treatment with a repurposed treatment
- 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

**Additional Proposal submission instructions can be downloaded at this link:**  
<http://bit.ly/cwrcalrfp>.

While repurposing clinical 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](mailto:clare@cureswithinreach.org) to discuss fit and/or submission options.

Cures Within Reach is circulating this RFP because there are thousands of pediatric rare diseases that 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 participate in a virtual event on June 10-11, 2021. More details about the event to come. 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.

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.**

**Important Funding Information:**

**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 20% Institutional Match. The 20% Institutional Match must be real dollars committed to the project, not in-kind efforts, 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; 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 (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% 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 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 of the REQUESTED PROJECT FUNDING and provide the \$100,000 of the EXISTING PROJECT FUNDING.*

**Cures Within Reach REQUESTED PROJECT FUNDING cannot be used for any Indirect Costs, although the 20% Institutional Match may be used for Indirect Costs.**

#### **Diseases/Conditions**

[Any pediatric 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 drug, device, nutraceutical or diagnostic](#)

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## Project Type

- Human Clinical Trial
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## Timeline

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

This research can take place anywhere, with a preference for US-based institutions, where good scientific research and clinical practices can be assured. This RFP is open to accredited academic and governmental research institutions and large group medical practices significantly involved with medical research. Start-ups, biotechs, pharmaceutical companies and medical device companies may also be eligible to apply. Contact Clare Thibodeaux, PhD at [clare@cureswithinreach.org](mailto:clare@cureswithinreach.org) to learn more about eligibility for companies.

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

This RFP seeks **clinical repurposing trials in any unsolved pediatric 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.

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## Restrictions

There are no other restrictions other than those described above.

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## Other Information for Researchers

If selected to participate in CureAccelerator Live!, the Principal Investigator will be required to participate in a virtual event which will be held on June 10 - 11, 2021. More details about the event to come.

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.

**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 repurposing clinical 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](mailto:clare@cureswithinreach.org) to discuss fit and/or submission options.

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### **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, organization or company proposing the research will match at least 20% of the REQUESTED PROJECT FUNDING. The Institutional Match must be real dollars committed to the project, not in-kind efforts, 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; or from any combination of these sources. All funding amounts are in US dollars.

Open to co-funding

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### **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.

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### **Due Date for Project Proposal Summary Submissions**

03-17-2021

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