

Juvenile Diabetes Cure Alliance

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What Must Change to Achieve a Practical Cure by 2025?

Conclusions:

- The goal of developing a Practical Cure by 2025 is unlikely to be met if the non-profits continue their current cure research funding paradigms.
- There are changes in research funding and other cure development strategies that the non-profits could implement immediately to meaningfully increase the probability of delivering a cure in this timeframe.
- Donors who seek a Practical Cure can take action to effect change by stipulating that their donations be used only for Practical Cure research and development.

Organizations of Focus:

American Diabetes
Association (ADA)

Diabetes Research
Institute Foundation
(DRIF)

JDRF

Joslin Diabetes Cen-
ter (Joslin)

TAKE ACTION NOW

Ensure it's for a Cure



✓ Specify for Practical Cure research.
Use our letter at www.thejdca.org/

✓ Call us for an advisory meeting at
212-308-7433

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This report outlines what must happen in order to deliver a Practical Cure for type 1 diabetes by 2025. First and foremost, meeting this goal requires a paradigm shift in how the major non-profits pursue cure development and fund research. The extensive recommendations presented in this report involve all of the major players: the non-profits, the research community, and donors.

Although a cure outcome by 2025 is not assured, the approach we delineate would attract new researchers and resources; yield new research approaches and innovations; accelerate cure progress; and reinvigorate donor and volunteer support. As a result, making the recommended changes would create the best chance to deliver a cure for those now living with type 1.

The Current Situation

After over forty years of cure development efforts, the non-profit establishment has failed to put a cure on the horizon. Their efforts lack focus in terms of research goals and funding practices. None of the major diabetes charities has set a time goal for a cure, and only one of the four, the DRIF, has adopted a definition of a cure. The absence of a time goal diminishes the urgency to develop a cure, while the lack of defined outcomes for cure research encourages open-ended exploration and misaligns valuable research resources. Put simply, the non-profits' allocation of cure research grants do not align with the intentions of cure donors. Too much funding is directed to research areas with no potential to deliver a cure, while some of the most promising cure research suffers from insufficient support.

One of the key findings of a recent JDCA survey is that **the donor community overwhelmingly prefers the pursuit of a Practical Cure over an Idealized Cure.**¹ A Practical Cure comprises a set of outcomes that can potentially be attained by 2025. It would not completely eliminate type 1 for patients, but it would permit them to live as if they did not have the disease, including an unrestricted diet, worry-free sleep, and diminished psychological burdens. (Please see Appendix A on page 5 for a complete description of a Practical Cure.) In contrast, an Idealized Cure for type 1 would theoretically eliminate the disease but would very likely require a significantly longer time to develop.

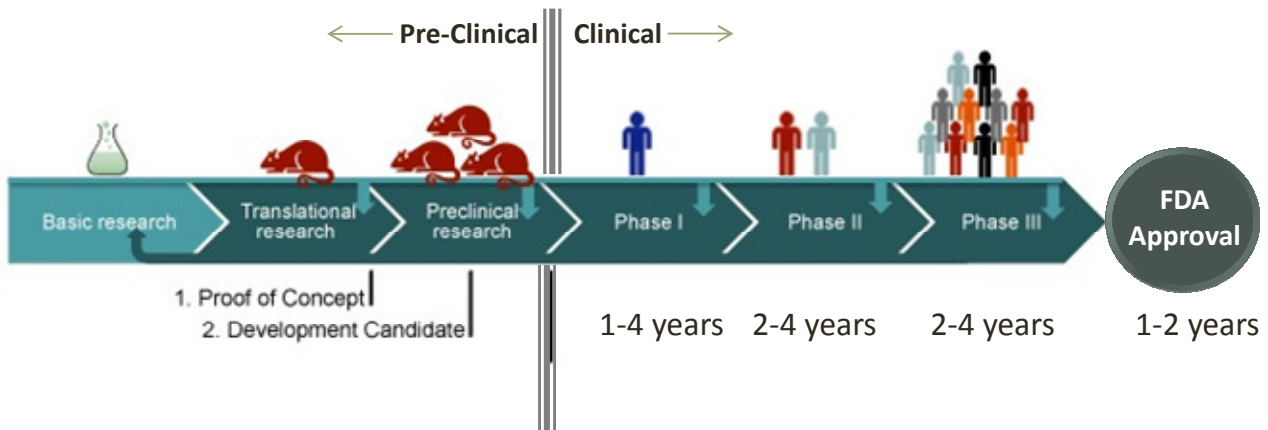
The JDCA believes that there is a moral imperative on the part of the non-profits to develop a cure as quickly as possible. The urgent need to produce a cure means that the non-profits must shift focus to Practical Cure research that is already underway. Existing research projects with the potential to deliver a cure for people who are now living with type 1 should receive elevated funding priority. Fully funding these projects would align with the intentions of current cure donors.

Time Considerations

The speedy completion of research is crucial in pursuit of a time-bound cure goal, as is a commitment to provide full funding for the most promising research projects. **Utilizing a time goal would deprioritize extensive areas of exploratory and ancillary research with no capacity to meet the targeted cure outcome so as to free up resources to support those projects that have the best potential to deliver a cure in our lifetime.**

The cure research and development timeline presented in the exhibit below can be used as a guide in estimating the total time necessary to complete the research process from the initial discovery phase through final FDA approval and the commercial availability of a cure. The chart covers the steps involved in the pre-clinical stage, which includes basic research through animal testing. It also lays out the likely time required to progress through the rigorous human testing stage of development and final FDA approval.

Exhibit A: Research Process from Project Initiation to FDA Approval



Source: California Institute for Regenerative Medicine; JDCA estimates, with contributions from Joshua Levy

Working backward chronologically from the right side of the chart, the mean estimated time required to commercialize a potential cure is ten years from the commencement of Phase 1 human clinical trials to final U.S. FDA approval. The time between today and 2025 is only slightly longer than the mean; hence, there is no time to waste.

Currently most Practical Cure research projects are in the pre-clinical stage of development, and not all of them are fully funded. Practical Cure projects collectively account for a very small percentage of the non-profits' total cure research budgets. A funding strategy that is more focused on promising near-term research would speed the projects' time to completion and advance viable pre-clinical research to human testing in time to meet the 2025 time goal. **Evaluating the pool of potential research projects against a time goal of 2025 implies that many exploratory projects will be rejected in favor of those that have the potential to deliver a cure in the near future.**

Action Steps for Non-Profits

To meaningfully increase the likelihood of developing a Practical Cure by 2025 requires an immediate paradigm shift in the non-profits' cure research funding strategies. Cure development projects are currently overwhelmingly skewed in favor of Idealized Cure research rather than Practical Cure projects. Of the 329 type 1 human clinical trials currently underway, only six projects, or two percent of the total, target a Practical Cure.² **The proportional allocation of funding needs to shift from Idealized Cure research in the direction of Practical Cure research if there is to be a reasonable chance of delivering a cure in time to benefit those now living with type 1.**

To meet this goal the non-profits also need to implement a Practical Cure research initiative that involves:

- adopting a Practical Cure definition and a time goal against which to strictly evaluate projects' viability and potential to meet these outcomes
- narrowing the research focus by prioritizing and fully funding the most promising projects that are designed to deliver the targeted cure outcomes within the time goal, regardless of whether the research is in the pre-clinical or human clinical trial stage of development
- de-emphasizing Idealized Cure research and exploratory research with less defined outcomes and/or time to completion that is irrelevant to individuals now living with type 1

- incentivizing management to deliver a cure and incentivizing researchers to design projects that endeavor to deliver Practical Cure outcomes
- developing objective metrics, including interim goals, to gauge tangible research progress
- reporting regularly on cure research and development performance

A fundamental change in the relationship between the non-profits and the research community also needs to occur. The non-profits need to become more proactive in soliciting the kind of projects they want to fund. They need to send a message to researchers that they seek and are willing to fund Practical Cure projects. This, in turn, will incentivize and encourage the research community to design and propose these types of projects.

The implementation of these strategic initiatives will have significant implications. It will strengthen cure development strategies and draw additional research resources and innovation to cure efforts. Adopting a Practical Cure research initiative would also reinvigorate support from jaded or disenfranchised members of the donor community and likely result in greater financial support for cure efforts.³

Action Steps for Donors

The best thing donors can do to drive change is to require that their donations be used for Practical Cure research. If you are a cure donor, explicitly stating how your money can be used will ensure that the non-profits use your donation in alignment with your intentions. The straightforward actions donors can take to significantly impact the chances of achieving a Practical Cure by 2025 include:

- Stipulating in writing that their contribution be used only for research that targets a Practical Cure. The JDCA provides a stipulation letter that may be downloaded from our website at:
<http://www.thejdca.org/wp-content/uploads/2012/11/Donor-Action-Letter.pdf>
Attaching this letter to your donation will ensure that the charity uses your contribution only for this type of research.
- Major gift donors may refer to the JDCA's Giving Guide on our website at:
<http://www.thejdca.org/wp-content/uploads/2012/11/Giving-Guide.pdf>
This document is a useful tool for donations that may involve special conditions or are structured to take effect over multiple years.
- Donors can also call the JDCA directly at (212) 308-7433 for a private consultation on structuring a Practical Cure research donation.

If you do stipulate for Practical Cure research, please let the JDCA know so that we may track this effort and monitor the non-profits' use of the stipulated donations.

Summary and Conclusion

In order to meaningfully increase the probability of developing a Practical Cure by 2025 a broad range of actions need to be implemented by the non-profits, researchers, and donor communities. Although a Practical Cure is by no means assured if these actions are taken, the non-profits have an obligation to maximize Practical Cure research opportunities and to better align their cure development efforts with the intentions of donors who seek a cure for individuals now living with type 1.

Several benefits would be derived from the non-profits adoption of a Practical Cure research initiative. New researchers and resources would emerge in the cure arena along with new projects and innovations, which would accelerate cure progress. It is also likely that the greater clarity and transparency associated with a Practical Cure research initiative would reinvigorate the donor community and garner greater financial support for cure research.

The non-profit organizations can implement the requisite changes of their own volition, or the donor community can drive the changes through their actions. Donors can bring about the change they seek by stipulating that their contributions be used only for Practical Cure work. Either way, the end result should be the same: more donor contributions will be directed to Practical Cure research and the chances of realizing a Practical Cure by 2025 will significantly improve.

Appendix A: JDCA Definition of a Practical Cure

A Practical Cure is outcome based and permits a 'like-normal' lifestyle



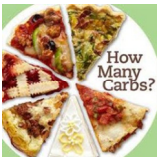
Minimal Monitoring

- ✓ Does not require blood glucose monitoring beyond once a week
- ✓ A1C levels 5-7%



Sleep Worry Free

- ✓ Allows patients to sleep care free



Free Diet

- ✓ Does not restrict a patient's diet
- ✓ Does not require carb counting



Minimal Side Effects

- ✓ Best case: Zero side effects
- ✓ Acceptable case: Insignificant side effects



Reasonable Meds

- ✓ If pharmacological, an easily managed regime



Fast Recovery

- ✓ If surgical, less than 72 hours recovery

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Endnotes

1. JDCA report, "Do Donors Feel That Practical Cure Research Is Important?," dated January 31, 2013.
2. JDCA report, "Update of Type 1 Human Clinical Trials That Target a Practical Cure," dated December 17, 2012.
3. JDCA report, "Do Donors Feel That Practical Cure Research Is Important?," dated January 31, 2013.

Analyst Certification

The JDCA analyst responsible for the content of this report certifies that with respect to each organization covered in this report: 1) the views expressed accurately reflect his own personal views about the organizations; and 2) no part of his compensation was, is, or will be, directly or indirectly, related to the specific views expressed in this research report.

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