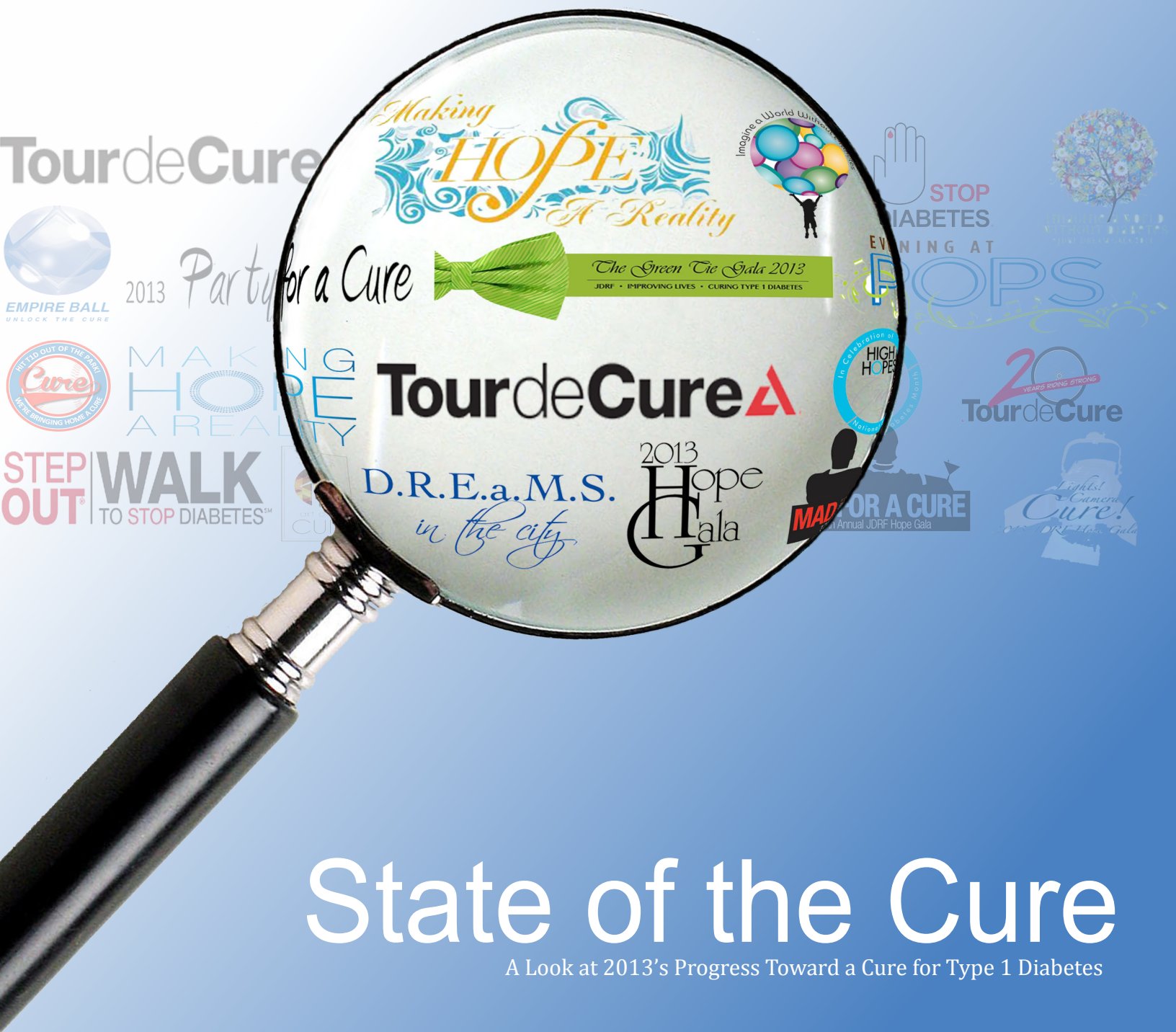
Juvenile Diabetes Cure Alliance

The Voice of the Donor For a Cure



State of the Cure

A Look at 2013's Progress Toward a Cure for Type 1 Diabetes

October 2013

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I. Introductory Letter

On behalf of everyone at the Juvenile Diabetes Cure Alliance, we are pleased to present *State of the Cure 2013*, our second annual review of progress toward a cure for type 1 diabetes. We've taken a comprehensive look back at cure-related developments in 2013 so that we can outline what needs to happen in the next twelve months in order to achieve a Practical Cure for type 1 by 2025.

The *State of the Cure* covers four main topics related to Practical Cure progress. First, we assess the significant impact that type 1 has on many Americans. Of particular note here is donors' strong support for a Practical Cure. Second, our review of Practical Cure research reveals that while medical science has made little material progress toward a type 1 cure in the past year, there are a few encouraging developments which can provide opportunities for future progress. Third, our analysis of managerial practices at the major diabetes non-profits finds a distinct misalignment between donor wishes and the non-profits' actual use of donor contributions. Finally, we recommend action steps that donors can take to drive substantive change in 2014.

At just a little over two years old, the JDCA remains deeply committed to bringing about a Practical Cure for type 1 diabetes by 2025. Over the past year we have published 17 reports and appeared in a variety of publications, including *The Wall Street Journal* and *The Chronicle of Philanthropy*. Based upon your input, we have also launched a high-impact donor advisory service and influenced millions of dollars in philanthropic giving for type 1.

The year ahead will take us into new territory. We will expand our coverage of institutions that take part in diabetes research, and amplify our advocacy for a Practical Cure through a larger media presence. We hope that you will use the *State of the Cure 2013* to inform and guide your philanthropic decisions throughout the upcoming year.

Thank you for your continued support of the JDCA and a Practical Cure for type 1.

Aaron Gorin
Director of Research Analysis

Phil Shaw
General Manager

II. The Need for a Type 1 Cure

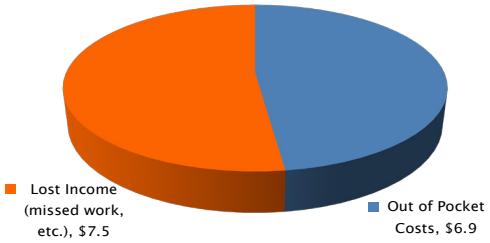
Over 5 million Americans are directly impacted by type 1 diabetes. At least one million are living with the disease, while another 4 million live in households that are managing type 1. [1]

Type 1 diabetes is an autoimmune disease in which the body cannot produce insulin because it has destroyed its pancreatic beta cells. Consequently, people with type 1 diabetes must manually administer insulin, either through injections or an insulin pump. Management is a daily and hourly task because blood sugar levels are affected by a number of often unpredictable factors, including meals, exercise, illness, and stress. It takes countless hours of planning and monitoring to maintain optimal A1c levels.

Mounting Costs

In addition to medical, emotional, and social burdens, type 1 also inflicts significant financial costs on individuals and the national healthcare system. Roughly $14.4 billion dollars is spent per year in the United States to provide medical care for type 1, funded primarily through insurance and government programs. This figure breaks down into $6.9 billion in out-of-pocket costs plus third-party payments that cover hospital and doctor visits, insulin, prescription drugs, medical supplies, home health provider services, and vision and dental care. The other $7.5 billion is attributable to lost income from missed work days and lower productivity. [2]

Exhibit 1:
Cost of Diabetes ($ Billions)



SOURCE: JDCA Research May 2013

Americans with diabetes incur medical expenses significantly higher than those without diabetes. Annual medical costs for a person with type 1 average around $9,900, which is nearly three times the average for people without type 1. In addition, managing type 1 becomes more costly later in life because the longer that one lives with the disease, the greater the chances of developing complications.[3]

Type 1 Diabetes Cure Progress

During the past half century there have been strong advances in treatments for type 1 but little tangible progress toward a cure. Innovations in the design of insulin pumps, more accurate testing tools, and faster acting insulin have made it easier to accurately monitor blood glucose levels and to administer insulin. Careful attention to diet and exercise, and disciplined daily management can lessen the risk of complications and extend life expectancy. But despite these treatment advances, a cure remains elusive.

The majority of cure research focuses on ideal solutions, or an "Idealized Cure." While some exciting avenues of research are being pursued, results from most of these pathways will require many decades to develop and are unlikely to be available for the generation of people who are currently living with type 1.

This report addresses the number one concern of donors: progress toward a cure for the current generation of people with type 1. The next section elaborates on the definition of a Practical Cure and the importance of a 2025 target date.

1 JDCA report, "The Size of the Population Impacted by Type 1 Diabetes," Aug 2013.
2 Betty Tao, et al, "Estimating the Cost of Type 1 Diabetes in the U.S.: A Propensity Score Matching Method," *PLos One* 5(7): 2010.
3 ibid.

III. What Is Practical Cure Research?

A Practical Cure for type 1 diabetes is any solution that 1) enables people with established type 1 to have a near normal lifestyle, and 2) has the potential to be widely available by 2025.

A Practical Cure is defined in the language of patients rather than medical science. Using non-scientific language keeps the objective simple and focused, and enables the greatest possible range of scientific solutions. Characteristics of a Practical Cure directly reflect the wishes and desires of the people living with type 1 and their families, which they have made clear over several years of interviews and survey research. There are three characteristics that set our definition of a Practical Cure apart.

1. Outcome based rather than scientific

In advocating for a Practical Cure, the JDCA does not favor any particular scientific approach to achieve the desired outcome. We track several research approaches that have the potential to deliver a Practical Cure by 2025. (The different Practical Cure research platforms will be discussed in Section VI.)

2. Potential to be widely available by 2025

The date is a target, not a promise. Donors overwhelmingly want to change the lives of people living with diabetes today. Setting a target date for a cure ensures that we are focusing on solutions that can impact this generation. Without such a time goal there is no mechanism to prioritize projects that have the best chance of delivering results sooner over projects that are more likely to produce results over a longer time frame.

3. Contrasts a practical solution versus a perfect or idealized one

An Idealized Cure, which is commonly presented as "the cure" for type 1, is predicated upon entirely eliminating the disease. In contrast, a Practical Cure is much wider in scope and includes any solution that frees a person with type 1 from the daily burden of the disease and the worry of complications. While a perfect solution would be welcomed, the scientific community has pursued one without success for over a century. Even now, Idealized Cure research is at such an early stage of conceptual development that it is unlikely to deliver a cure in time to benefit anyone who is currently living with type 1. The next section illustrates that donors expect a cure to be available much sooner.

Exhibit 2:
Practical Cure Characteristics

Minimal Monitoring

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

Free Diet

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

Sleep Worry Free

- ✓ Allows patients to sleep care free

Minimal Side Effects

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

IV. Donor Attitudes

The JDCA has conducted eight surveys of the type 1 diabetes donor community over the last two years to gauge donors' expectations, priorities, and attitudes about cure progress. In all cases we sought to accurately track and disseminate donor and community sentiment in the hope of improving alignment between donor wishes and the non-profits' funding priorities.

The surveys were conducted by a third party and were carefully monitored to follow best practices of market research and survey design. These surveys collectively pooled answers from over 2,000 respondents. The results were statistically significant, and the margin of error never exceeded 10%.

Donor Expectations

The JDCA surveys provide three key learnings: 1) donors expect a cure in the near future; 2) they would prefer to fund projects that could produce a cure sooner rather than later; 3) they are willing to give to Practical Cure research if that option was made easily available. In fact, nine out of ten donors prioritize funding Practical Cure research over funding Idealized Cure research.

Many people with type 1 diabetes have been hearing that a cure is imminent since they were diagnosed, even if that diagnosis was decades ago. Expectations are stoked by the flurry of news stories about reversing type 1 diabetes in mice, research progress touted as "on the cusp of major breakthrough," and fundraising events that promise to find a cure. As shown in Exhibit 3, seven out of ten donors believe that a cure will actually arrive in the next ten years, and nearly all donors want a cure in the next decade.

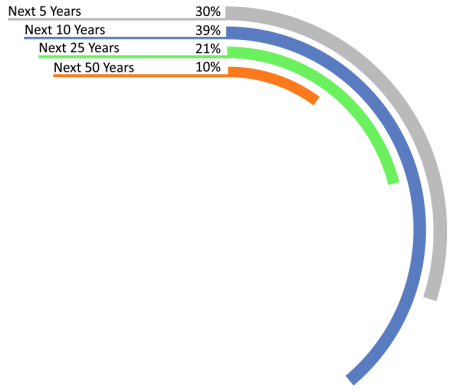
Given the value and expectation placed on delivering results within the next decade, it is no surprise that donors nearly universally state that they would much prefer to fund projects that may deliver results in a shorter timeline than projects that are on a much longer time horizon.

Opportunity for Non-Profits to Expand Cure Funding

Donors value the pursuit of a Practical Cure to such a degree that they are willing to give expressly for this purpose. Exhibit 5 shows that eight out of ten *active* donors state that they would either "definitely" or "probably" donate specifically to Practical Cure research if that option was made easily available. The concept of a Practical Cure is even appealing enough that a full third of *lapsed* donors who have not given in several years would consider once again becoming active contributors to the diabetes non-profits if their funds were applied to Practical Cure research.

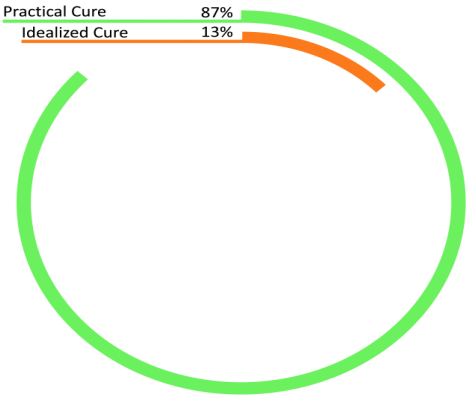
Identifying projects that target the outcomes of a Practical Cure and committing donor contributions to that purpose will drive an increase in donations. Active donors are prepared to contribute for a Practical Cure, and a good percentage of lapsed donors will be motivated to renew giving as well. Unfortunately, the non-profits only pursue Practical Cure research to a limited degree, as we discuss in the next section.

Exhibit 3:
When do you think a type 1 cure will arrive?



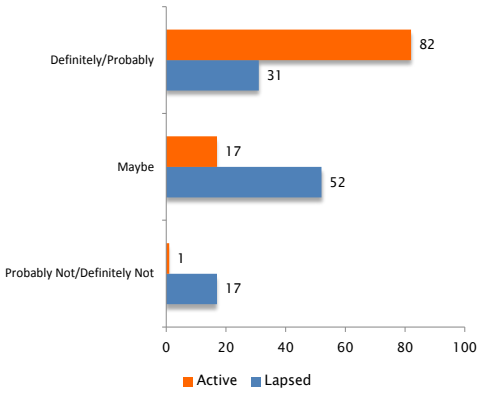
SOURCE: JDCA proprietary survey, Donor Attitudes and Behaviors, May 2013

Exhibit 4:
Which cure outcome would you prefer?



SOURCE: JDCA proprietary survey, Donor Attitudes and Behaviors, May 2013

Exhibit 5:
Would you donate to a Practical Cure if you had the option? (% of Respondents)



SOURCE: JDCA proprietary survey, Donor Attitudes and Behaviors, May 2013

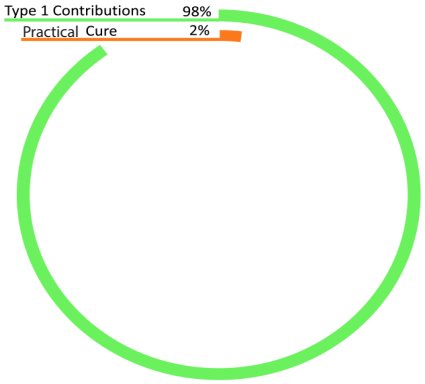
V. Funding for Practical Cure Research

The JDCA finds that only two cents of every donor dollar were directed to Practical Cure research projects, as shown in Exhibit 6. We base our analysis of the non-profits' cure research funding on publicly available information, including each organization's annual report, Form 990, audited financial statements, official website, and other publications.

The chart below demonstrates the percentage of donor contributions that each non-profit allocates to Practical Cure research. Notably, the ADA and Joslin devote none of their budgets to Practical Cure research. While JDRF funded the most Practical Cure research in absolute dollars, its $6 million allocation represents only 3% of total donor contributions. The DRIF's $3 million allocation represents a larger 27% of its donor contributions.

Exhibit 6:

PC Research Funding as a % of Total Type 1 Donor Contributions



SOURCE: JDCA Report: "Only 20% of Funds Raised for a Cure Allocated to Cure Research," Sept 2013

Exhibit 7:

Donor Contributions Directed to Practical Cure Research

Organization	Donor Contributions (mm)	Practical Cure Research (mm)	PC Research (% of contributions)
ADA	$178	$0	0%
DRIF	$11	$3	27%
JDRF	$196	$6	3%
Joslin	$13	$0	0%
Total	$398	$9	2%

SOURCE: JDCA Report: "Only 20% of Funds Raised for a Cure Allocated to Cure Research", Sept 2013

A transformative shift in the cure research strategies of the non-profits is needed to create a more balanced research portfolio. If the non-profits are to spend donor money according to donor wishes, a higher percentage of donor contributions should be allocated to Practical Cure research.

> "Only two cents of every donor dollar was directed to Practical Cure research."

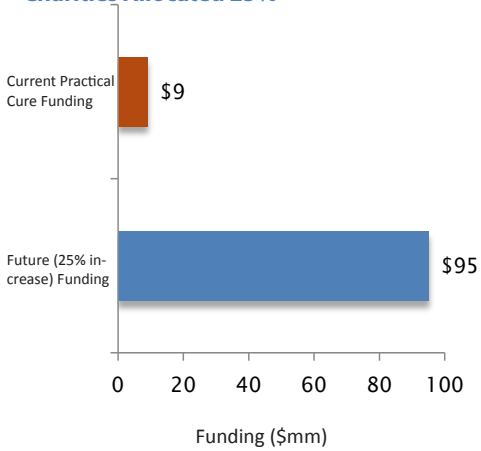
Focusing a larger portion of the total research portfolio on Practical Cure projects increases the probability of developing a Practical Cure by 2025. We recognize that research targeting complications, glucose control, and prevention has value to donors and we are not suggesting that such research should be abandoned. Ideally, the JDCA would like to see all of the non-profits direct at least 25% of donor contributions to a Practical Cure research initiative. Of the $398 million in donations to the four major non-profits, only $9 million was directed to Practical Cure research projects (2%). Increasing that from 2% to 25% would translate to nearly $100 million in total Practical Cure research funding.

We believe that while the 25% target is a long term goal, Practical Cure funding is so low right now that any budgetary shift toward Practical Cure work would have a significant impact. Each incremental 1% of overall donor contributions, or $4 million, would mean an almost 50% increase from the current level of funding for Practical Cure research, which underscores the importance of starting the shift in capital allocation priorities as soon as possible.

Until the non-profits proactively create a Practical Cure Research initiative, **donors have the ability to increase funding for this work by stipulating that their donation be used only for Practical Cure research.** In the following section we outline the various research platforms that donors who seek a Practical Cure could support.

Exhibit 8:

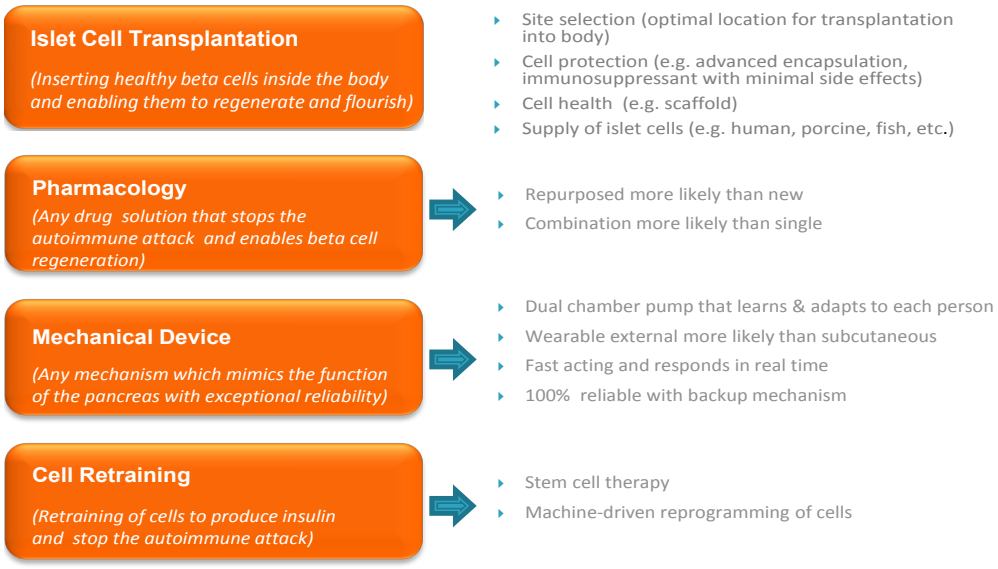
Donor Contributions and PC Research if Charities Allocated 25%



VI. Practical Cure Research Platforms

As shown in Exhibit 9 below, the JDCA has identified four current research approaches that may result in a Practical Cure: islet transplantation, pharmacology, mechanical devices, and cell retraining. As mentioned in Section II, the JDCA is neutral with respect to scientific approaches. The fundamental differences between the platforms concern only the method by which the targeted outcomes are achieved rather than the viability of the scientific approach. The JDCA does not forecast the likelihood of any one particular platform's success.

Exhibit 9:
Practical Cure Platforms



SOURCE: JDCA Research

Among the four current approaches, human clinical trials are currently underway for research projects that target a Practical Cure via islet cell transplantation, cell retraining, and pharmacology. We discuss these projects in Section VII. There are no medical devices that currently meet all of the criteria for a Practical Cure, but a future iteration may do so, as medical devices can potentially progress to market faster than pharmaceutical approaches.

Given the ten or more years that it could take for a project to advance through pre-clinical and clinical testing and then receive final U.S. FDA approval, a potential Practical Cure would need to advance to at least the pre-clinical stage of development fairly soon in order to be market ready by 2025. In the next section, we review the major human clinical trials that have the potential to achieve a Practical Cure.

VII. Practical Cure Research in Human Clinical Trials

In 2013, the JDCA reviewed over 300 active type 1 diabetes human clinical trials. The overwhelming majority of these trials focus on glucose control, complications, and general observations of patient care outcomes. Only six projects have the potential to deliver a Practical Cure. A summary of these six trials and their status in 2013 is shown in Exhibit 10.[1]

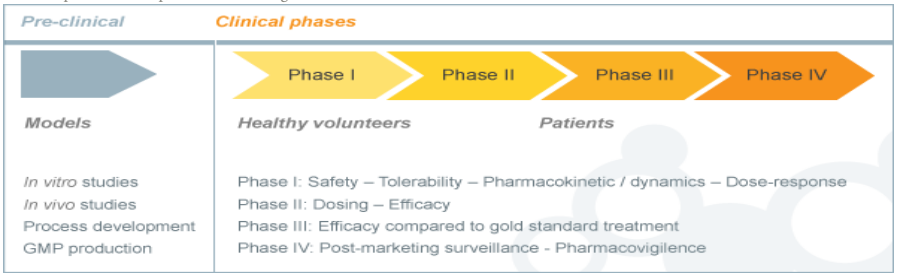
While the number of Practical Cure research projects in human clinical trials remained the same as in 2012, there are some notable updates. The ATG/GCSF project was dropped from the list after ATG failed to show a prolonged increase in c-peptide production in its phase II clinical trial. We added Viacyte to this year's list and intend to actively monitor its ongoing phase I stem cell trial. Among the candidates from last year's list, most are still enrolling or are actively engaged in trials, aside from the LCT/Diabecell announcement that its commercial launch is now expected in 2018, not 2016.

Exhibit 10:
Practical Cure Projects in Human Clinical Trials

Project Name	Location	Description	Update	Phase
LCT/DIABECELL	LCT living cell technologies	Transplanted porcine islets that are micro-encapsulated	Completed Phase I/IIa trial in Russia. Performed 16 of the 30 patient implants through 6/30/13. Commercial launch delayed from 2016 to 2018.	II
Monolayer Cellular Device	Cliniques universitaires Saint-Luc	Beta cell encapsulation approach that uses human islets	Enrollment scheduled to complete by December 2013.	I
Tianhe: Stem Cell Educator Therapy	TIANHE STEM CELL BIOTECHNOLOGIES	An individual's blood is treated with stem cells which has the effect of reversing autoimmunity and stimulating beta cell growth	Published Phase I/II data or July 2013 demonstrating safety & efficacy. Enrolling for the international multi-center Phase II.	II
Sitagliptin/ Lansoprazole	SANFORD RESEARCH	Drug combination aimed at both stopping the autoimmune attack and stimulating beta cell growth	24 month Phase I trial began in August 2012 and remains ongoing. Results are expected by year-end 2014.	I
Faustman: BCG Vaccine	faustmanlab.org Researching a cure for type 1 diabetes	Drug that kills disease-causing autoimmune cells and restores pancreatic beta-cell function through regeneration	Recruiting for Phase II trial and awating funding. As of September 15th, lab had raised $16m of the $25m needed.	II
Viacyte	MU	Stem-cell-derived pancreatic cells are encapsulated in a drug delivery system implanted under the skin	Phase 1 stem cell therapy trial underway, using progenitor cells developed from human embryonic stem cell line to produce insulin.	I

SOURCE: JDCA Research, Company Data

1. A brief representative explanation of the stages of FDA clinical trials:



VIII. Emerging Practical Cure Research

Beyond the Practical Cure projects in human clinical trials outlined in Section VII, the JDCA has identified several research projects that align with the Practical Cure platforms, but are only in pre-clinical trials at this time. Some of these projects are funded by the major non-profits, while others receive support from smaller research centers around the world.

Among the major non-profits, only JDRF and the DRIF fund Practical Cure research, while the ADA and Joslin do not. The JDRF supports Practical Cure projects at numerous research institutions, whereas the DRIF only funds the Diabetes Research Institute's BioHub, which has a stated focus of developing a "biological cure" for type 1 diabetes. The BioHub's holistic, integrated approach to developing a cure across scientific platforms is a promising strategy that could be widely replicated across the non-profit and research community. However, we still look for improvements in the DRIF's transparency regarding its actual funding allocations, and the status and prioritization of research projects.

Within the smaller research centers, we have identified institutions around the globe that are researching Practical Cure solutions across the spectrum of scientific platforms. This list in the exhibit below is not exhaustive; we intend to actively monitor these and other emerging programs in the coming year.

Exhibit 11:
Emerging Practical Cure Research at Select Institutions

Platform	JDRF Funded	DRI Funded	Other Funded
Islet Cell Transplanation	Vanderbilt University, Emory University, University of British Columbia	University of Miami (BioHub)	Chicago Diabetes Project, Universitair Ziekenhuis Brussel, New York Presbyterian, Emory University
Cell Retraining	University of British Columbia		University of Missouri
Pharmacology	University of Florida		Perle Biosciences, Stanford University

SOURCE: JDCA Research

IX. Fundraising Methods and Messaging

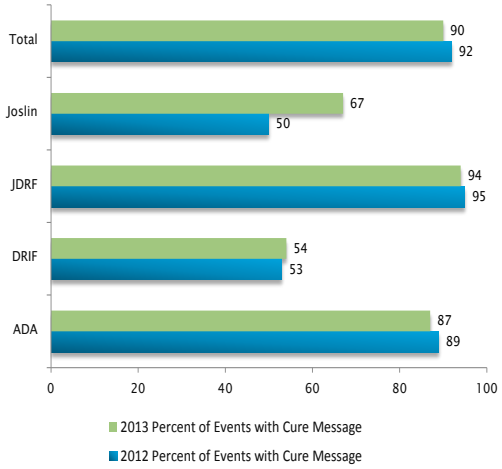
The JDCA reviewed over 550 fundraising events in the United States in 2013, including all major events organized by the four diabetes non-profits plus major third-party-organized events. Similar to 2012 levels, cure messaging is used both explicitly and implicitly to promote 90% of these fundraising events in 2013, ranging from walks and galas to bike rides and golf outings, as shown in Exhibit 12. Among the four non-profits, the two biggest fundraisers (JDRF and the ADA) rely the most on cure messaging, at 94% and 87% of their events respectively.

Examples of direct messaging include JDRF's "Walk to Cure Diabetes" and "Ride to Cure Diabetes," or the ADA's "Tour de Cure" and "Step Out: Walk to Stop Diabetes." Examples of implicit messaging includes JDRF's "Dream Gala," and "Promise Gala."

When comparing each organization's reliance on cure messaging relative to their funding of cure research, we found a substantial gap between event messaging and funding allocations. As shown in Exhibit 13, 90% of total events use the cure message, but only 18% of overall donations are used to fund cure research. We note that this 18% figure is a self-categorized measure of cure research funding, as defined by the non-profits, and does not represent funding for a Practical Cure, which we peg at 2% overall.

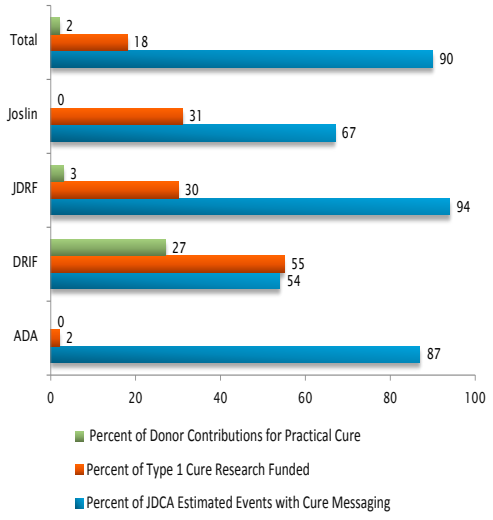
The next section reviews how corporate governance practices at the non-profits could increase accountability for using donor contributions to fund the kind of research donors want to fund.

Exhibit 12:
Events Using Cure Messaging (%)



SOURCE: JDCA Report, "Only 20% of Funds Raised for a Cure Allocated to Cure Research," Sept 2013

Exhibit 13:
Cure Messaging vs. Cure Funding (%)



SOURCE: JDCA Report, "Only 20% of Funds Raised for a Cure Allocated to Cure Research," Sept 2013

X. Corporate Governance

Strong corporate governance, which includes many executive and board level management practices, is one of the best indicators of how effectively an organization is run. The JDCA recently rated the corporate governance practices of the major non-profits after an extensive review of publicly available information and interviews with organization executives. A summary of the findings and recommendations is presented below (full report at www.thejdca.org).

Our review of corporate governance involves several main areas: 1) strategy and organization direction; 2) transparency and timelines of communications; 3) accountability of management against achievement of objectives; and 4) resource utilization. In a for-profit company, the board of directors has a fiduciary duty to ensure that these areas are executed in a way that is consistent with Securities and Exchange Commission guidelines, which were established to protect and uphold the rights of shareholders of the company.

There remains a great opportunity for non-profits to benefit from the governance practices of for-profit entities. Although non-profit organizations do not have shareholders, they do have donors and stakeholders who provide the capital that enables these organizations to operate. The JDCA advocates that treating the donors as shareholders would create a win-win for both parties, changing the relationship between donor and non-profit from transactional to collaborative.

Aggregate Findings

The JDCA's second annual review of governance concludes that the non-profits'managerial practices are substantially below the best practices of public companies. Exhibit 14 below outlines key learnings in the four STAR ratings areas:

Exhibit 14:
Summary of STAR Rating

Area	Finding	Suggestions
Strategic Direction	✓Generally the strongest area ✓Donor input not generally sought before shifts in strategy or introduction of new initiatives	✓Donors 'vote' on major strategic shift or new intiative. Publish outcome. ✓External validation of effectiveness towards mission. Publish outcome.
Transparency and Timeliness	✓Few timely reports on progress of specific research projects versus milestones. Info presented in aggregate, if at all. ✓Delays in reporting of 990s and annual reports	✓Publish all financials, annual report, and 990 within 3 months of year end. ✓Publish bi-annual comprehensive research updates.
Accountability/ Management	✓Rewarded more for fundraising achievements than actual progress against a cure ✓None have a compensation program that is directly linked to type 1 cure progress	✓Tie performance metrics and compensation directly to progress towards a cure.
Resource Allocation	✓Actual utilization generally not aligned with donor interests. Majority of contributions not actually used for cure research.	✓Align broad allocation of budget with donor wishes and key fundraising promises. Publish breakdown of research funding.

XI. The Need for Donor Action

You – the readers of this report – are the key to giving us the best chance of reaching a Practical Cure in time to change the lives of people now living with type 1. This section briefly outlines a few actions that we can all take to make a difference. Please keep in mind that the JDCA does not solicit donations for itself and offers this and all of our reports throughout the year as a free service to the community. Our sole purpose is to achieve a Practical Cure by 2025. You can help.

The main finding of this report is that despite some promising developments, not enough material progress was made toward a Practical Cure during 2013. The bad news is that unless we change the paradigm by which research projects are selected, funded, and prioritized, we may not see a breakthrough anytime soon. The good news is that we can build on the promising developments in 2013. This year we saw the launch of the DRI's BioHub, learned that several smaller research centers are making progress on what may be key components of a Practical Cure, and have begun to see Practical Cure language seep into the non-profits' vocabulary. But we need to do more.

Challenging an established approach is never easy, and gaining a priority position for Practical Cure research is no exception. But together we can bring about a paradigm shift that gives precedence to the pursuit of a Practical Cure.

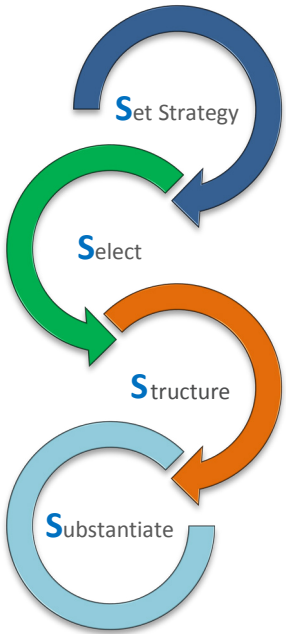
Specifically, we can take two significant steps to improve the outlook for a cure:

Join Voices. By becoming a Juvenile Diabetes Cure Alliance member you add your voice to a rapidly growing community of over 10,000 like-minded people who all want to see a Practical Cure become a top priority.

Apply Financial Incentive. Ensure that each donation you make is applied to a Practical Cure by attaching a letter to your contribution that stipulates that the funds be used solely for Practical Cure research. A template for stipulations is available at www.thejdca.org/donor-tools. There is no more powerful way to accelerate progress to a Practical Cure than to specifiy exactly how we want our contributions to be used.

Exhibit 15:
Cure Donor Advisory Service

The JDCA provides a free advisory service for donors interested in giving with maximum impact. It covers the 4S's of good giving:



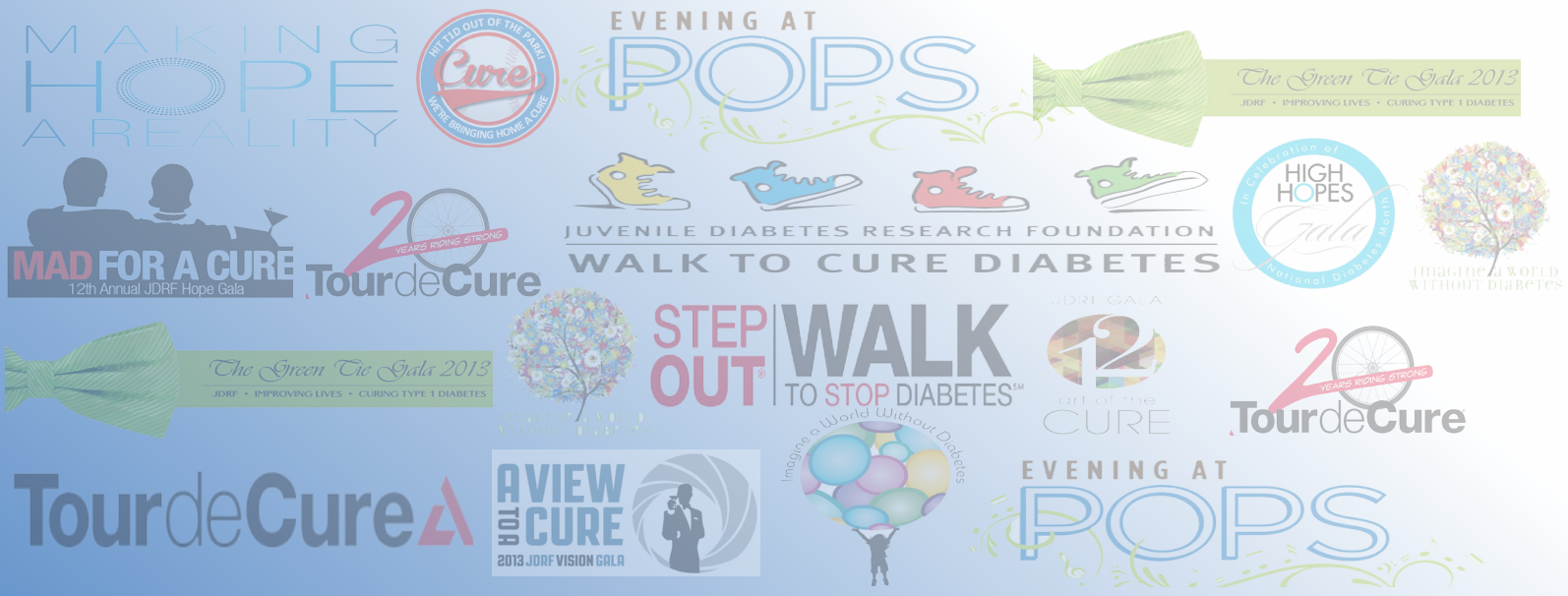
Strategy:
What are my goals and objectives for giving?

Select:
Given what I want to achieve, who is the best recipient for my gift?

Structure:
What is the best way to go about making my gift? One year? Multi-year? Restricted? Contingent?

Substantiate:
Was my gift actually used as I wanted it to be used?

For more information contact
Cara Murphy
212-308-7433



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Website: thejdca.org

About Us

The Juvenile Diabetes Cure Alliance is a non-profit organization that unites individuals who seek a Practical Cure for type 1 diabetes by 2025. We work with donors to ensure that their charitable contributions fund research with the best chance of delivering a Practical Cure. We conduct independent research that analyzes topics related to type 1 cure development with a focus on the four major diabetes non-profits and report our findings to Alliance members and the donor community at large.