The Voice of the Donor for a Cure

Juvenile Diabetes Cure Alliance

May 1, 2012

Peter Miselis, CFA

Director of Research Analysis

212.308.7433

pdm@thejdca.org

Is Type 1 Cure Research Funding Focused Enough?

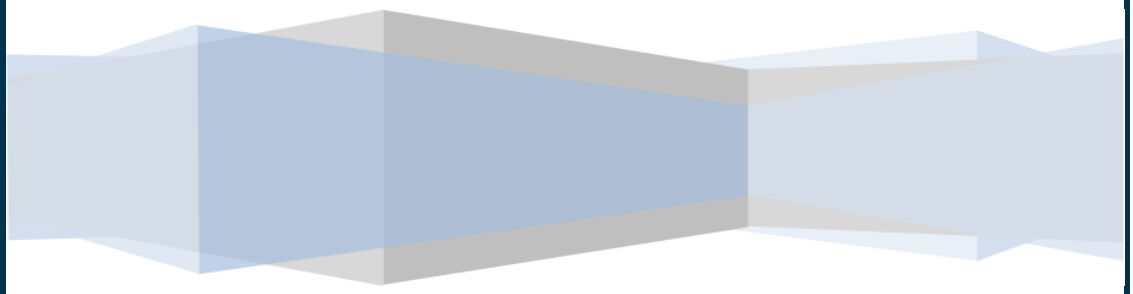
Conclusions:

→ The four charities fund a large number of research projects every year, yet none of them target a specifically defined type 1 cure

→ Applying a broad-based funding approach has not resulted in a cure for type 1, nor is a cure in sight

→ The process by which charities fund type 1 cure research has been ineffective and has resulted in very few human clinical trials that have the potential to deliver a Practical Cure

→ We believe that a far more effective cure development strategy would be to aggressively pursue a limited number of promising research opportunities that have the potential to deliver a defined cure outcome



Our Mission:

To direct donor contributions to the charitable organizations that most effectively fund research with the goal of delivering a type 1 Practical Cure by 2025.

Organizations of Focus:

American Diabetes Association (ADA)

Diabetes Research Institute Foundation (DRIF)

JDRF

Joslin Diabetes Center (Joslin)

In recent reports we estimated that generous donor contributions to the four major type 1 diabetes (type 1) charities totaled $370 million in 2011, but that only 21%, or two of every ten dollars raised, was allocated to type 1 cure research grants.[1] The remaining dollars (79% of the total) were allocated to non-cure research grants, including type 2 research; non-research activities such as education/information/awareness/advocacy; and overhead. This report examines the organizations' approach to funding type 1 cure research and proposes an alternative strategy that could accelerate the development of a type 1 cure.

The charities' approach to developing a type 1 cure is to pursue a large number of projects across a range of research categories, an approach that can be characterized as broad and shallow. The application of a broad strategy indicates that the organizations direct funding without clearly defined goals. Funding a wide range of projects, including those with no potential to deliver a type 1 cure, diverts resources from those projects deemed to have the highest cure potential, thus diminishing the chances of achieving a type 1 cure.

The pursuit of so many projects spreads resources thin by parceling funds into numerous small grants that cannot sufficiently support the intense research necessary to deliver a cure. Funding requirements for many pre-clinical trials can easily be in the hundreds of thousands of dollars. Conducting Phase I or Phase II human clinical trials requires millions of dollars. In contrast, many of the charitable foundations' grants provide funding of $50,000 or less. For example, the ADA awarded 59 research grants (21% of its total) of less than $50,000 each in 2010.[2]

The following table lists the charity, the number of research grants it funded in 2011, and its dollar allocations to research grants. Research grants include three areas: cure, glucose control, and complications. Donors should also bear in mind that JDRF and the DRIF are dedicated solely to type 1 whereas the ADA and Joslin have a dual-focus on both type 1 and type 2.

	Number of Research Grants	Research Grants (millions)
JDRF	459	$116
ADA	276	$33
DRIF	35	$7
Total	770	$156

Sources:

JDRF research grant number from the Diabetes Journal article on the "Juvenile Diabetes Research Foundation."[3] The dollar amount is from 2011 financial statements.

ADA number of research grants is a JDCA estimate and is estimated to be flat with 2010. Research Grant dollar amount is from 2011 financial statements.

DRIF number of projects is a JDCA estimate and Research Grant dollar amount is from 2011 financial statements.

Joslin is excluded from the table because the number of research projects is not available. However, Joslin spent $31 million on Research Grants in 2011, bringing the total Research Grant allocation for the four organizations to $187 million.[4]

Funding large numbers of new research grants annually is a practice that has been in place for the past several years, and for possibly far longer. We estimate that over 2,000 separate research grants were funded by the three organizations over the five years 2007-11.[5] The total number of research projects funded prior to the peak of the 2008 financial crisis was significantly higher than in the latest year. This funding philosophy would suggest that increased donor contributions would be applied to a greater number of projects rather than to deepening the focus on the most promising opportunities.

The 2011 estimated total of 770 grants by the three organizations (JDRF, ADA, and DRIF) is a very large number. On the surface, funding a substantial number of research grants appears impressive. However, the majority of these grants fund small or unrelated projects, are basic or exploratory in nature, or do not target a specific cure outcome. Such a funding strategy has been an ineffective approach to curing type 1 for the following reasons:

- → **The process of funding thousands of research projects over a forty year period has not resulted in a cure for type 1, nor is any cure in sight**
- → Funding a wide range of projects that may include basic or exploratory research has resulted in very few human clinical trials that could potentially deliver a Practical Cure. Only five human clinical trials out of over 300 that are underway have the potential to deliver a Practical Cure.
- → **No clear goals have been established with respect to defining a cure or adopting a cure-by date for type 1. These are fundamental to the development of a type 1 cure.**
- → Total available resources are spread thin over a broad range of projects, thereby diverting funding from the projects deemed to have the best potential to deliver a cure

<u>If the current process of funding type 1 cure research is ineffective, is there a better alternative?</u>

A narrow and deep funding strategy that pursues fewer and better funded research programs could concentrate resources on the most promising cure opportunities. We believe that a Practical Cure will derive from overwhelming, urgent, and persistent support for a select group of research projects which have the potential to meet clearly defined goals. Pursuing a narrow and deep approach is particularly relevant to the development of a Practical Cure for type 1 because the complexity of the challenge and the length of time required to deliver a cure require a very focused research effort. Combining clear end goals with a targeted strategy maximizes the chances for cure development. Such an approach would:

- → Focus on projects that target specific cure-related goals, including a cure definition and a cure-by date
- → Ensure that the most promising projects are aggressively pursued
- → Adequately fund projects that have potential to deliver a Practical Cure
- → Accelerate the timeline to delivering tangible cure results
- → Avoid potential duplication of research efforts

Notable examples of very focused strategies that met ambitious goals and objectives include the following:

- → In 1997 Apple was an unfocused company that produced many products and generated poor operating results. During that year, under the leadership of Steve Jobs, a program was initiated that resulted in a 70% reduction in the number of products and focused the company on just four key product areas. With a narrow focus, the company has since excelled in sales and profit growth and is now the world's most valuable company.[6]
- → In 1961 President Kennedy set the goal of landing a man on the moon before the end of the decade. Given this clearly defined goal and the relatively short time table, NASA succeeded at landing men on the moon in 1969, and repeated its success with five subsequent manned moon landings. For the forty years since then, NASA has not had such clarity of mission. During this time frame hundreds of billions of dollars have been spent on space programs, yet NASA has not accomplished anything close to the universally acknowledged momentousness of putting a man on the moon in 1969.[7]
- → Throughout the 1950s-60s many companies researched a wide range of potential cholesterol lowering agents (statins). In 1971, after reviewing the field of research, Dr. Akira Endo focused his time and effort on fungi as a high potential source for cholesterol-lowering agents. After obtaining mixed results from one substance, Endo maintained his focus on fungi and discovered another agent that produced superior results. Continued success in statin development then attracted the interest of Merck, which eventually obtained U.S. FDA approval for the first commercial statin in 1987. This was the beginning of what became an annual multi-billion global market that includes drugs such as Lipitor.[8]

Summary/Conclusion

The major type 1 charities' approach to funding type 1 cure research has been ineffective in delivering a type 1 cure. The organizations fund a large number of research projects every year, however, many of these grants are unrelated and relatively small in size. They often support basic, exploratory, or idealized research that does not target specific cure goals. In fact, none of the charities have even adopted a definition of a cure. As a result, after forty years of research, no Practical Cure for type 1 is on the horizon.

The JDCA believes that a far more effective cure development effort would:

- → Define a cure, such as the Practical Cure definition set forth by the JDCA[9]
- → Set a cure-by date, which imparts a greater sense of urgency to cure development
- → Narrow the focus of cure research to projects that have the potential to deliver the defined cure by the target date
- → Aggressively pursue and adequately fund research that has the potential to achieve specific cure outcomes within a time frame that is relevant to individuals that are living with type 1

Juvenile Diabetes Cure Alliance

14 East 60th Street
Suite 208
New York, NY 10022

Phone: 212.308.7433
E-mail: info@thejdca.org
www.thejdca.org

1. Please see the JDCA reports "Review of Expense Categories of the Major Type 1 Non-Profits," dated March 8, 2012, and "Shrinking Funding of Type 1 Cure Research by the Four Major Non-Profits," dated April 5, 2012.

2. ADA 2010 Form 990

3. Insel, R. A., Deecher, D.C., & Brewer, J. (2012). Juvenile Diabetes Research Foundation: Mission, Strategy, and Priorities. *Diabetes*, 61, 30-35.

4. Joslin 2011 Audited Financial Statements

5. Charity data and JDCA estimates

6. Isaacson, W. (2011). *Steve Jobs*. New York: Simon and Schuster.

7. NASA Langley Research Center Office of Public Affairs. (1992, December). The Rendezvous That Was Almost Missed: Lunar Orbit Rendezvous and the Apollo Program. Retrieved from: http://www.nasa.gov/centers/langley/news/factsheets/Rendezvous.html.

For NASA budget figures see: http://www.hq.nasa.gov/office/hqlibrary/find/newnasadoc.htm

8. Endo, A. (2010, May). A historical perspective on the discovery of statins. *Proceedings of the Japan Academy, Series B Physical and Biological Sciences*, 86, 484-493. Retrieved from: http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3108295/?tool=pubmed.

9. Please see the JDCA "A Practical Cure vs. An Idealized Cure for Type 1," dated October 20, 2011.

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.

Other Disclosures