Testimony by

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At the Hearing entitled:

"Redefining Reality: How the Special Diabetes Program is Changing the Lives of Americans with Type 1 Diabetes"

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Introduction

Chairman Collins, Ranking Member Casey, and Members of the Committee, thank you for welcoming all of these delegates who are in the nation's capital for the JDRF Children's Congress, and for giving me the opportunity to testify before you today.

All of the delegates sitting before you live with type 1 diabetes, or T1D, a disease caused by an autoimmune response that damages the cells that make insulin. I too live with type 1 diabetes, and have since the age of 13. So does my brother Stephen, who was diagnosed back in 1977 at the age of 3.

Before the discovery of insulin in the early 1920s, type 1 diabetes was universally fatal. Its discovery saved millions of lives; however, insulin is not a cure. Type 1 diabetes requires constant management, 24 hours a day, 7 days a week, 365 days a year, to avoid dangerous high and low blood sugars and devastating complications.

Diabetes impacts every aspect of life. These children do so much hard work to manage their diabetes – they are truly amazing and their parents are too. Each of these families can share countless stories about how difficult T1D can be, as can mine.

Special Diabetes Program Making a Tremendous Difference

But we can also tell you that today, we can better manage this disease and live healthier lives than ever before, because of research funded by the Special Diabetes Program – due to your leadership.

On behalf of all of us, I want to thank you. The Special Diabetes Program (SDP) is making a tremendous difference in our lives and in our hopes for the future. Your strong bipartisan support for the SDP has led to numerous research breakthroughs, transforming lives and bringing us closer to our ultimate goal of curing this disease.

I am a scientist by training and have spent the last 15 years at JDRF, the world's largest charitable funder of T1D research, becoming its President and CEO in April.

In my time at JDRF, I've seen firsthand how the combination of federal diabetes research funding and JDRF's private investment constitutes one of the most effective public-private partnerships focused on curing a chronic disease.

Allow me to share some of the highlights.

Progress in Artificial Pancreas Systems

Just last month, exciting clinical results were released showing that a new artificial pancreas system which both doses and withholds insulin at appropriate times helped people with T1D maintain more consistent, and healthier, blood glucose levels. The clinical study, supported by the SDP, found that the advanced hybrid closed-loop technology resulted in more time in range – fewer highs and fewer lows – with no severe hypoglycemic events, normalized overnight glucose levels, and importantly, less burden for people with T1D.[1]

Also in recent months, the U.S. Food and Drug Administration has approved the first continuous glucose monitor (CGM)[2] and insulin pump[3] that can work interoperably, and needed software is not far behind. This will create the opportunity for people with diabetes to select the component devices of their system, tapping into tremendous innovation without having to put together a 'do it yourself' system.

This progress all builds on the success of the first artificial pancreas system which came on the market in 2017,[4] several years earlier than expected thanks to your leadership, Senator Collins, and innovative research supported by the SDP. This system was discussed extensively at a hearing in this Committee two years ago and has had a major positive impact for our community.[5]

Thus, thanks to the SDP, we will soon have access to multiple FDA approved artificial pancreas systems, enabling people with T1D and their doctors to *choose* the system that works best for them. This choice is critically important, because we know that people with diabetes achieve better outcomes when they can choose the tools that are right for them in managing their disease.[6] That's why at JDRF, while our goal is to cure T1D, we also are fighting for ways for people with T1D to stay healthy until that day. That entails supporting research to develop next-generation technology, strongly

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[1] Kwon, J, Brown A. "Tandem's Control-IQ System Increases Time in Range and Lowers A1C in People with Type 1 Diabetes", *diaTribe*, June 28, 2019. Accessed at https://diatribe.org/tandems-control-iq-system-increases-time-range-and-lowers-a1c-people-type-1-diabetes.

[2] U.S. Food and Drug Administration, "FDA authorizes first fully interoperable continuous glucose monitoring system, streamlines review pathway for similar devices," March 27, 2018. Accessed at https://www.fda.gov/news-events/press-announcements/fda-authorizes-first-fully-interoperable-continuous-glucose-monitoring-system-streamlines-review.

[3] U.S. Food and Drug Administration, "FDA authorizes first interoperable insulin pump intended to allow patients to customize treatment through their individual diabetes management devices," February 14, 2019. Accessed at https://www.fda.gov/news-events/press-announcements/fda-authorizes-first-interoperable-insulin-pump-intended-allow-patients-customize-treatment-through.

[4] U.S. Food and Drug Administration, "MiniMed 670G System", July 26, 2018. Accessed at https://www.fda.gov/medical-devices/recently-approved-devices/minimed-670g-system-p160017s031.

[5] U.S Senate Special Committee on Aging, "Progress Toward a Cure for Type I Diabetes: Research and the Artificial Pancreas" July 26, 2017 hearing. Accessed at https://www.aging.senate.gov/hearings/progress-toward-a-cure-for-type-i-diabetes-research-and-the-artificial-pancreas.

[6] American Association of Clinical Endocrinologists and American Association of Diabetes Educators to UnitedHealthcare, May 23, 2019. Accessed at https://www.diabeteseducator.org/docs/default-source/advocacy/provider-letter-to-uhc-ceo--aace-aade.pdf?sfvrsn=2.

advocating for affordable insulin and other diabetes management tools, and adamantly opposing health plan policies that limit choice.

Progress in Immunotherapies

Another promising area of research that has been advanced by funding from the SDP and JDRF involves the use of immunotherapies to delay or prevent the onset of T1D. Last month, the clinical trial network, TrialNet, published results in the *New England Journal of Medicine* that found the immunotherapy drug teplizumab can delay the onset of T1D for an average of two years in children and adults.[7]

I cannot emphasize enough how important this finding is.

Every day without T1D matters. A delay in onset is likely to have long-term benefits for glycemic control, and the reduction in acute and long-term complications would have a tremendous impact on the daily lives of our community and to our overall health system.

Moreover, this study raises the possibility that in the future targeted immunotherapies could mitigate or even cure T1D. While this study was a phase 2, not a phase 3 trial, it shines the light on a promising potential pathway.

This progress is thanks to your leadership and Congress' foresight to invest in multi-year funding for SDP research.

Progress in Eye Therapies

When I was first diagnosed with T1D 35 years ago, vision loss was almost a given in T1D.

Today, thanks to the SDP, and investments by JDRF and the private sector, there are multiple therapies available to help preserve and even improve sight. These advances make the difference between being able to see well enough to drive – or not.

The SDP also filled a critical research gap by funding a comparison of three drugs for the treatment of diabetes-related eye disease.[8] The results help patients, clinicians, insurers and policymakers make better informed decisions about targeted treatment. This comparison likely would not have happened in the private sector.

[7] Herold, K, Bundy, B, Long, S, Bluestone, J. "An Anti-CD3 Antibody, Teplizumab, in Relatives at Risk for Type 1 Diabetes", *NEJM*, June 9, 2019. Accessed at https://www.nejm.org/doi/10.1056/NEJMoa1902226.

[8] The Diabetic Retinopathy Clinical Research Network, "Aflibercept, Bevacizumab, or Ranibizumab for Diabetic Macular Edema" N Engl J Med 2015; 372:1193-1203. Accessed at https://www.nejm.org/doi/10.1056/NEJMoa1414264.

More Work to Be Done

Yet, while these developments have altered the science of T1D, and reduced the burden of the disease so that people have better outcomes, we are here today because there is still important work to be done.

The Special Diabetes Program must continue to invest in innovative immunotherapy and beta cell research, our most promising cures pathways. To make progress towards curing T1D, we need to understand why the immune system goes awry, and how we can eliminate these immune attacks. Unlocking these answers would have implications across numerous diseases, from multiple sclerosis and rheumatoid arthritis to cancer. At the same time, we need to test additional novel approaches to prevent or slow the onset of T1D in those most at-risk to develop it.

We need to capitalize on the amazing scientific promise in beta cell therapies. Thanks to the SDP, the Human Islet Research Network was established to organize collaborative research about beta cell regeneration and replacement. It is helping us better understand how beta cells, the cells in the body that produce insulin, are damaged in people with T1D so we can find strategies to protect or replace them and ultimately cure the disease.

We need to better understand the triggers for T1D. The Environmental Determinants of Diabetes in the Young study (TEDDY) has screened more than 425,000 children and enrolled 8,600 children determined to be at-risk of developing T1D to understand what environmental factor or factors trigger the onset of the disease.

This SDP-funded study is more than halfway to completion. Information on diet, infections, and other exposures is being analyzed from children who are progressing or now have full disease onset to help us understand what causes T1D so strategies can be developed to prevent it altogether. The extensive data gathered from this study will benefit research into other autoimmune diseases as well. It's crucial that we see this study to completion.

At the same time, we need to reduce the burden from kidney and heart disease.

Kidney disease is a life-threatening complication of T1D that creates a significant personal and economic burden. In 2015, end-stage renal disease cost Medicare $34 billion. If new therapies could lower ESRD rates by 50 percent, Medicare would save more than $51.6 billion in 10 years.[9]

A promising SDP-funded trial is testing whether a generic medication may halt or slow the progression of early kidney disease in people with T1D. This trial is yet another

[9] Winn, A, Skandari, R, O'Grady, M, and Huang, E. "Potential Medicare Savings of Reduced End Stage Renal Disease in Patients with Diabetes," February 2019. Unpublished white paper.

example of how the SDP is filling critical gaps as pharmaceutical companies have no incentive to test for new uses of a generic drug.

Heart disease is also a significant burden for people with diabetes. In 2014, 1.5 million people with diabetes were hospitalized for major cardiovascular disease.[10] Overall, people with diabetes face a twofold increase in heart disease compared to those without diabetes.[11] However, people with T1D face an even greater risk.[12]

Additional research is needed to evaluate implications for treatment, such as conducting studies to determine if certain drugs have protective effects against heart disease in people with T1D.

The Importance of Your Leadership

Together, this is just a snapshot of all the Special Diabetes Program is – and could be – doing to help Americans living with T1D. Senators, let me say that this research is too important to have an expiration date.

We know what happens when this funding is put on hold. In 2017, when the SDP renewal was delayed, there were real implications. Within the TrialNet clinical trial network, enrollment was postponed in a promising prevention trial. By the time funding was in place and enrollment began, some people who would have been eligible had since developed full onset T1D and could therefore no longer enroll.

With continuous funding, their diagnosis potentially could have been delayed. We cannot risk slowing the momentum we've gained, and allow this to happen again.

We are so grateful to all the Senators here today for their support of the SDP.

We are particularly grateful for the outstanding leadership of the Senate Diabetes Caucus Co-Chairs, Senators Collins and Shaheen, who championed a bipartisan letter in support of the SDP which 68 Senators sent to the Senate leadership in May.

We are very pleased that in June, S. 1895, the Lower Health Care Costs Act, was approved by the Senate Committee on Health, Education, Labor, and Pensions with a five year renewal of the SDP.

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[10] Centers for Disease Control and Prevention, "National Diabetes Statistics Report, 2017". Accessed at https://www.cdc.gov/diabetes/pdfs/data/statistics/national-diabetes-statistics-report.pdf.

[11] Sarwar N, Gao P, Seshasai SR, Gobin R, Kaptoge S, Di Angelantonio E, Ingelsson E, Lawlor DA, Selvin E, Stampfer M, Stehouwer CD, Lewington S, Pennells L, Thompson A, Sattar N, White IR, Ray KK, Danesh J. "Diabetes mellitus, fasting blood glucose concentration, and risk of vascular disease: a collaborative meta-analysis of 102 prospective studies." *Lancet*, 2010. Accessed at https://www.thelancet.com/journals/lancet/article/PIIS0140-6736(10)60484-9/fulltext.

[12] de Ferranti, Sarah D et al. "Type 1 diabetes mellitus and cardiovascular disease: a scientific statement from the American Heart Association and American Diabetes Association." *Diabetes Care* vol. 37,10 (2014): 2843-63. Accessed at https://care.diabetesjournals.org/content/37/10/2843.article-info.

Conclusion

As you can see, the Special Diabetes Program is making a real difference in the lives of people with T1D.

We need Congress to enact a five year renewal of the program to keep researchers working, without interruption.

And, we need your continued leadership, so that when these children are my age, they can say they “used to have T1D”.

Thank you, and I’d be happy to take any questions.