Testimony by

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"Accelerating Breakthroughs: How the Special Diabetes Program is Creating Hope for those Living with Type 1 Diabetes"

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Chair Murray, Vice Chair Collins, Senator Shaheen, and Members of the Committee, thank you for welcoming all our amazing JDRF 2023 Children's Congress Delegates, their guardians, and our special guests. Also, thank you for allowing me to testify before you today. I am honored to be here and to be joined by Dr. Rodgers and three of our amazing JDRF advocates to highlight the important progress advanced by the Special Diabetes Program and our hope for the future.

I am Dr. Aaron J. Kowalski, Chief Executive Officer for JDRF, the leading global organization harnessing the power of research, advocacy, and community engagement to advance life-changing breakthroughs for type 1 diabetes, or T1D. A scientist by training, I have been fortunate to work for JDRF and on behalf of countless individuals and families impacted by T1D for the past 19 years, four of which in my current role.

Like every delegate in this room and far too many people across the world, my family and I are impacted by T1D. My family's experience dates to 1977 when, out of the blue, my younger brother, Stephen, was diagnosed at the age of 3. My own T1D diagnosis followed when I was 13.

Grappling day-to-day with T1D was difficult then and it still impacts every aspect of life today. But we have good news to share.

Because of your steadfast leadership, Senators Collins and Shaheen, and the strong bipartisan support in Congress, the Special Diabetes Program, or SDP, has fundamentally changed what it means to live with diabetes, put new life-changing therapies in our hands, and brought us closer to cures. The SDP's sister program, the Special Diabetes Program for Indians (SDPI), is yielding similar significant and impactful results. We must keep this momentum going so we can capitalize on the progress to date and realize cures.

Diabetes – a Costly and Burdensome Disease

First, let me start by sharing a bit about type 1 diabetes. T1D is an autoimmune disease in which a person's pancreas stops producing insulin, a hormone that enables a person to utilize energy from food. T1D lasts a lifetime, and people with T1D must take insulin to live. Type 2 diabetes, also known as T2D, is a metabolic disease. With T2D, the body still produces insulin but cannot use it effectively. While T1D and T2D are different, the costly and burdensome complications are the same.

Today, more than 37 million Americans have diabetes and 96 million have prediabetes[1]. The disease affects people of all ages and races across the country. The complications that result from diabetes are significant -- ranging from heart disease and strokes to lower-limb amputations.

Due to the disease's breadth and impact, about one in four healthcare dollars and one in three Medicare dollars are spent treating people with diabetes. The total cost of diagnosed

[1] Centers for Disease Control and Prevention. National Diabetes Statistics Report website. https://www.cdc.gov/diabetes/data/statistics-report/index.html. Accessed July 6, 2023.

diabetes in the United States in 2017 was a staggering $327 billion, with $237 billion spent on direct medical costs and another $90 billion on reduced productivity[2]. Another astounding fact is that the economic costs of diabetes, adjusted for inflation, increased by 26 percent from 2012 to 2017[3]. As more people develop diabetes, these costs will be even more significant.

Fortunately, Congress had the foresight to address the growing magnitude of the challenge years ago. With bipartisan support, the SDP and SDPI were created as part of the Balanced Budget Act of 1997 and have been strongly supported by both chambers of Congress and both parties ever since.

JDRF Partnering with the Federal Government

Before I share some of the exciting breakthroughs resulting from the SDP, I want Congress to know that JDRF's private funding works hand in hand with SDP funding to ensure that the most important and promising research projects have the support they need. JDRF accelerates the path to cures by raising funds and allocating them to T1D research and therapy development, as well as by leveraging our expertise and leadership to bring in additional funding and supporters.

I am proud to say that JDRF has invested more than $2.5 billion in research funding since our founding in 1970. And through direct research investments and partnerships within the public and private sectors, including through the SDP, we helped raise $483 million for T1D research and therapy development in FY21. This partnership between the public and private sectors is what it takes to deliver major advances for T1D research.

Special Diabetes Program Accelerating Breakthroughs

Since we held our last Children's Congress in 2019, there has been remarkable progress that gives us even more hope for a brighter future. These are just a few of the many advancements that are exciting to the T1D community where the SDP played a role.

- **First Disease-Modifying Therapy Brought to Market**

  The SDP enabled the creation of TrialNet, the largest clinical network for T1D. TrialNet conducted the clinical trials that led to the November 22 U.S. Food and Drug Administration (FDA) approval of Tzield, the first disease-modifying therapy for T1D. This therapy can delay onset for nearly three years; others to delay and ultimately prevent onset are in the research pipeline.

  A recent study examined how Medicare spending would be impacted by delaying the development of T1D by 3, 5, and 10 years. For the current and future population living with diabetes, different amounts of time delaying diagnosis are expected to generate a

[2] American Diabetes Association. Economic costs of diabetes in the US in 2017. Diabetes Care. 2018 May;41(5):917–928.
[3] American Diabetes Association. Economic costs of diabetes in the US in 2017. Diabetes Care. 2018 May;41(5):917–928.

range of Medicare savings that build over time. Cumulative savings accrued to Medicare range from $1.6 - $3.0 billion within 10 years. By the 25-year mark, the Medicare savings reaches $5.9-$16.6 billion[4]. These analyses do not account for the costs associated with delaying diabetes development.

- **Advances in Cell Therapies**

  A recent exciting development in diabetes research is the June 2023 FDA approval of the first cell therapy for adults with T1D. The approval is specifically for adults unable to maintain average blood glucose levels due to repeated severe episodes of low blood glucose levels or hypoglycemia. This therapy, with the use of immunosuppression, takes deceased donor islets and places them into people with T1D. This is an important first for the diabetes community, as it is a therapy that allows some patients to go without any injected or infused insulin for several years. Clinical trial data resulting from the SDP-supported Clinical Islet Transplantation Consortium contributed to this pivotal step toward identifying cures.

- **Artificial Pancreas Systems**

  Another area of progress that is a real game changer for people with T1D is in the field of technologies to better manage blood glucose levels. An artificial pancreas (AP) system consists of an insulin pump, a continuous glucose monitor (CGM), and a computer program called an algorithm that allows the pump and CGM to talk to each other to give the right amount of insulin at the right time – much like the pancreas does in people without T1D. AP systems are not perfect, but they allow people to think about their diabetes less and have better blood glucose control, which improves the quality of life of those impacted by T1D and helps to mitigate costly and burdensome long-term diabetes-related complications.

  SDP-funded research laid the early groundwork for developing AP systems. In fact, SDP funds contributed to the first fully automated insulin-dosing system being made available to patients in 2017, some five to seven years earlier than expected. Positive results from SDP-supported clinical trials since then have led to other FDA-approved systems and next-generation AP devices that have outperformed first-generation devices in adolescents and young adults.

  The majority of the delegates with us here today have AP systems. The SDP funded the clinical trial that led to the FDA approval of the system I use, and my blood glucose control is much more manageable because of it. I am excited to see what comes next for AP systems, which will keep us healthier until we have cures.

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[4] Winn, A, Skandari, R, O'Grady, M, and Huang, E. "Potential Medicare Savings of Delaying Type 1 Diabetes Onset," May 2023. Unpublished white paper.

Opportunities for Future Funding

In the early years of SDP funding, we were excited to discover a few new genes that might unlock clues to understand the origin of this disease better, and now we have transformational therapies and management tools that are improving lives and bringing us closer to our ultimate goal of cures for this disease. While the opportunities before us are abundant, these are just a few examples in need of funding with the next SDP renewal.

- **Fund Long-Term Research Programs and Clinical Trials**

  Continued support of the SDP would keep several long-term T1D-oriented research programs and clinical trial networks running strong, enabling them to continue their work which has yielded so many exciting and promising discoveries. For example, The Environmental Determinants of Diabetes in the Young (TEDDY) study of 8,600 children, enrolled at birth and being followed until they are 15 years old, seeks to understand which environmental factors trigger or protect against T1D onset. Information on diet, infections, and other exposures is being analyzed from children who are progressing toward - or now have - full T1D onset. Results to date indicate that there are multiple pathways leading to full T1D onset. Further investigation is needed to develop strategies to prevent the onset of the disease, ranging from a vaccine to specific dietary changes. The data collected from this study could also benefit other autoimmune diseases, such as celiac disease.

- **Protect Heart and Kidney Health for Those with T1D**

  SGLT2 inhibitors protect heart and kidney health for people with type 2 diabetes but are not yet approved for people with T1D. There is an immediate need to develop evidence-based strategies to ensure these drugs can safely be used by people with T1D, which could reduce costly and devastating impacts of heart and kidney disease.

  A recent study shows that for the current population living with T1D, lowering End Stage Renal Disease rates by 50% is expected to generate Medicare savings that build over time. At the ten-year mark, the cumulative savings to Medicare are $5.1 billion. By the 25-year mark, Medicare savings reach $13.6 billion[5]. These savings estimates do not incorporate individuals who will be diagnosed in the future and therefore are conservative.

[5] Winn, A, Skandari, R, O'Grady, M, and Huang, E. "Potential Medicare Savings of Reduced End Stage Renal Disease in Patients with Diabetes," May 2023. Unpublished white paper.

- **Investigate Cardiovascular Disease in People with T1D**

  Cardiovascular disease (CVD) in people with T1D is not nearly as well studied as in people with T2D despite the fact that it is a major cause of death in people with T1D. There is a major unmet need to identify the mechanisms that promote earlier development of CVD in people with T1D, to learn and develop new interventions to prevent and treat it.

Long-term, Uninterrupted SDP Support Needed

The critical research supported by the SDP requires long timeframes to achieve optimal results. A long-term reauthorization would allow the National Institute of Diabetes and Digestive and Kidney Diseases to carefully plan the use of funds, which are directed toward new and ongoing clinical trials that take multiple years to complete.

Technology development also takes time. The noteworthy progress in developing artificial pancreas and other glucose management technologies has been spurred by long-term SDP support, as I noted earlier. A short-term reauthorization would make it much more difficult to undertake long-term planning in these areas.

For the scientists who conduct the research, long-term, sustained funding is incredibly important. With the uncertainty of sustained funding, they may pursue other research opportunities elsewhere. The assurance of long-term funding is also important to attract new talent to this field and to maintain a pipeline of future generations of scientists to conduct T1D research.

Federal Funding for NIH, FDA, and ARPA-H

While the SDP is a key component in our collective efforts to accelerate T1D research breakthroughs, three key agencies – the National Institutes of Health, the recently formed Advanced Research Projects Agency for Health and the Food and Drug Administration – are also vital to speed the advancement of research and access of new therapies for people with T1D. We ask that this committee support these agencies with the highest funding levels possible.

Insulin Affordability

While the focus of this hearing is on the Special Diabetes Program, research progress, and hopes for the future, it is important to note that people with diabetes need access to affordable insulin until we have cures. As you have heard from your constituents and our many advocates, the current drug pricing system in the United States does not work for countless people who need insulin to survive.

Over a 10-year period, the cost of insulin increased threefold[6]. Currently, insulin can cost between $175 and $300 per vial and up to $1,000 per month. Studies have shown that these inflated costs can cause up to one-quarter of people with diabetes to skip or ration their insulin[7], potentially leading to costly medical emergencies, severe complications, or death.

We are seeing real progress in addressing this critical issue through actions taken by governments, health plans, and insulin manufacturers. Medicare now caps the cost of insulin at $35 per month due to the Inflation Reduction Act. Earlier this year, Lilly, Novo Nordisk, and Sanofi announced reductions in the cost of their insulin. And the Civica Insulin Project, of which JDRF is a funder, is on track to produce the first of three biosimilar insulins for $30 per vial and $55 for a box of five pens. They intend to submit to the FDA next year.

However, we must do more. We need Congress to take the next step this year. JDRF urges Congress to pass the bipartisan INSULIN Act of 2023, led by Senators Collins and Shaheen, which establishes a $35 per month insulin copay cap for people with commercial insurance and includes other provisions that would make insulin more affordable for everyone, regardless of insurance status.

Conclusion

As you can see, the Special Diabetes Program is indeed improving and saving lives and creating hope for those with type 1 diabetes. We have come so far since I was diagnosed as a teen and even since our last Children's Congress in 2019. The future is bright for all these delegates with us today and the countless others across the country who are impacted by T1D, with continued support for the SDP and your help.

Senators Collins and Shaheen, we are tremendously grateful for your leadership. And members of this committee, we so greatly appreciate your support. I know many of you are among the 60 Senators who signed the letter, led by Senators Collins and Shaheen, supporting the SDP and its renewal. Thank you!

I urge you all to vote in favor of S. 1855, led by Senators Collins and Shaheen, which was approved by the Senate Health, Education, Labor, and Pensions Committee. The bill would increase the current funding of the SDP from $150 million to $170 million for two years and include nearly $43 million more to extend the program through December 2025. The current funding expires at the end of this September, so I urge you to act quickly. And please support next steps on insulin affordability and do that promptly as well. We are counting on you.

Thank you again for the opportunity to testify. I am happy to answer any questions.

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[6] Centers for Disease Control and Prevention. National Diabetes Statistics Report website. https://www.cdc.gov/diabetes/data/statistics-report/index.html. Accessed July 6, 2023.

[7] Centers for Disease Control and Prevention. National Diabetes Statistics Report website. https://www.cdc.gov/diabetes/data/statistics-report/index.html. Accessed July 6, 2023.