Testimony
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A Future Without Type 1 Diabetes: Accelerating Breakthroughs and Creating Hope

Statement of

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Chair Collins, Vice Chair Murray, and Members of the Committee, as Director of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), I thank you for your invitation to testify at this hearing on type 1 diabetes. On behalf of NIDDK and the other Institutes and Centers of the National Institutes of Health (NIH) within the U.S. Department of Health and Human Services (HHS), I am honored to be here today to update you on recent scientific advances and future research opportunities in type 1 diabetes and its complications, including research supported by the *Special Statutory Funding Program for Type 1 Diabetes Research* (*Special Diabetes Program*).

Diabetes takes an enormous personal and economic toll on our country, but we are making great strides in reducing that burden through biomedical research. NIDDK supports the majority of diabetes research at NIH, which includes studies on both prevention and treatment of type 1 diabetes, type 2 diabetes, gestational diabetes, and diabetes complications. Funding from the *Special Diabetes Program* is part of the NIH investment in diabetes research, and it enables the agency to expand type 1 diabetes research beyond what is possible with our regular appropriations and to conduct clinical trials that follow more patients for longer periods of time than would otherwise be feasible. NIH's efforts have been complemented by our research partners—the U.S. Food and Drug Administration (FDA); the Centers for Disease Control and Prevention (CDC); academic institutions; and charitable, professional, and patient advocacy groups such as Breakthrough T1D (formerly JDRF), the Endocrine Society, the American Diabetes Association (ADA), and the Leona M. and Harry B. Helmsley Charitable Trust.

Through the invaluable support of Congress, the tireless work of our researchers, and the dedication of our clinical research volunteers, we have made significant progress toward our goals of understanding, preventing, treating, and ultimately curing type 1 diabetes—with more progress on the horizon.

Improving the Lives of People with Type 1 Diabetes

Type 1 diabetes is an autoimmune disease, where the immune system attacks and destroys the insulin-producing beta cells in the pancreas. People with type 1 diabetes (or their caregivers) must monitor their blood glucose (sugar) levels, administering insulin as needed depending on their food intake, physical activity levels, and other factors. This is an enormous burden, and despite constant vigilance, keeping blood glucose levels consistently in a healthy range can be a challenge. For over 25 years, NIDDK has deployed *Special Diabetes Program* funds toward research with high potential to improve the health of people with and at risk for type 1 diabetes, both in the short and long term.

One important way that we gauge our success is to determine whether our research findings are directly reaching and benefiting people with type 1 diabetes. I am pleased to report that NIH-supported research advances have dramatically improved the health and quality of life of people with and at risk of the disease, as well as their families. For example, *Special Diabetes Program*-supported research has culminated in the first ever FDA-approved preventive therapy for type 1 diabetes; the first ever FDA-approved cellular therapy for type 1 diabetes; and multiple new FDA-authorized artificial pancreas devices for children and adults that have become the standard of care for many people with type 1 diabetes.

While we are heartened by this progress, we also recognize there is more to achieve. The SEARCH for Diabetes in Youth study, supported by CDC and NIDDK, has reported that type 1 diabetes incidence is increasing in youth under age 20 in the United States.[1] It is imperative that we combat this increase by identifying the factors driving it and developing new prevention approaches for this younger population. Additionally, while the artificial pancreas has reduced the burden of managing disease, people with type 1 diabetes and their families want that burden reduced further – they want a cure. NIDDK remains committed to supporting critical research areas to achieve our longer-term goals, which include helping to identify potential cures.

Improving Glucose Management and Quality of Life

For people with type 1 diabetes, keeping blood glucose levels within a healthy range is a key step to maintaining good health and preventing or delaying the development of long-term complications of the eyes, kidneys, nerves, heart, and other organs. NIDDK, with *Special Diabetes Program* funds, has prioritized the development of new tools and technologies to help people control their blood glucose levels with less burden.

The *Special Diabetes Program* has contributed to this area by supporting the development of glucose management technologies, including artificial pancreas systems that automate insulin delivery in response to blood glucose levels. I am pleased to report that NIDDK and *Special Diabetes Program*-supported research contributed to the development or testing of most of the currently available commercial artificial pancreas devices. One of these devices was also recently FDA cleared for adults with type 2 diabetes who are taking insulin,[2] demonstrating the far-reaching benefit that *Special Diabetes Program*-supported research is having on daily management of disease for all individuals with diabetes.

We are extremely pleased that the research investment supported by the *Special Diabetes Program* has made such a significant and positive impact on the lives of people with the disease and their families. While we celebrate this progress, we are also looking ahead to developing the next generation of artificial pancreas systems. New technologies can enable the development of devices that are less invasive and burdensome, while improving durability, adaptability, reliability, and accuracy. We also have opportunities to augment these devices with artificial intelligence (AI)/machine learning (ML) tools, to optimize their function for individual users.

Restoring Beta Cell Function

Technologies for managing type 1 diabetes are important tools for alleviating the burden of this disease, but they are not a cure. Finding a biological cure for type 1 diabetes that restores the body's ability to produce insulin and regulate blood glucose levels is another major, long-term goal of NIDDK- and *Special Diabetes Program*-funded research. I am pleased to report that decades of NIH- and *Special Diabetes Program*-supported research, including research conducted by the Clinical Islet Transplantation Consortium, an effort co-led by NIDDK and the National Institute of Allergy and Infectious Diseases (NIAID), paved the way for the June 2023 FDA approval of a cellular therapy—islet transplantation—for adults with type 1 diabetes whose

[1] https://pubmed.ncbi.nlm.nih.gov/36868256
[2] www.fda.gov/news-events/press-announcements/fda-roundup-february-28-2025

disease cannot be managed using current therapies. This landmark achievement represents the first ever FDA-approved cell therapy for treating type 1 diabetes.[3]

While this therapy represents a major step forward in providing a cure for those with type 1 diabetes, islet transplant recipients must take long-term immunosuppressive medicines to prevent the body from attacking the transplanted islets. These medicines may cause serious side effects. Additionally, there is a shortage of cadaveric islets available for transplantation. These limitations underscore the need for additional research to develop more broadly available cell replacement therapies. Researchers are currently looking for ways to prevent islet transplant rejection without the need for long-term immunosuppression, such as by encapsulating islets with a material that protects them from immune system attack.[4] Scientists are also using knowledge from the study of other diseases and applying it to type 1 diabetes. Based on the success of CAR-T therapy for cancer treatment, scientists in the *Special Diabetes Program*-supported Human Islet Research Network (HIRN) engineered immune cells that protect transplanted islets from immune system rejection without tamping down the protective parts of the immune system in a mouse model.[5] This finding represents an exciting approach to advance islet transplantation, and it could prove useful for other organ transplantations, expanding the impact of the *Special Diabetes Program*.

In parallel with research to protect transplanted cells from immune attack, scientists are also vigorously studying ways to generate and preserve sufficient numbers of islets for transplantation. This research has already yielded success—cell products and encapsulation technologies developed in part with *Special Diabetes Program* support are now being tested in industry clinical trials. HIRN researchers are investigating how beta cells develop and mature with the goal of one day allowing for the production of new beta cells. HIRN researchers reported a groundbreaking discovery that pancreatic alpha cell dysfunction precedes beta cell loss in people who have a single type 1 diabetes autoantibody (a protein made by the immune system that signals an early stage of type 1 diabetes). This finding is paradigm changing and defines a new, distinct, early stage of type 1 diabetes. Additional studies of this early stage could pave the way for developing clinical interventions to protect or replenish beta cells.[6]

Additionally, HIRN has developed new models of type 1 diabetes, including sophisticated mouse models that mimic key features of human disease and three-dimensional laboratory models of human pancreatic islets, that will enable novel studies of pathophysiology and serve as platforms for pre-clinical testing of new therapies. Continued research through HIRN and other efforts holds promise to yield knowledge necessary to further develop cell replacement therapy as a type 1 diabetes cure.

Preventing Type 1 Diabetes Initiation and Progression

Preventing type 1 diabetes has been a long-standing goal of NIH-supported research. Toward this goal, research supported by the *Special Diabetes Program* culminated in the 2022 FDA approval of teplizumab as the first therapy that can delay the onset of stage 3 type 1

[3] www.fda.gov/news-events/press-announcements/fda-approves-first-cellular-therapy-treat-patients-type-1-diabetes
[4] https://pubmed.ncbi.nlm.nih.gov/37106151; https://pubmed.ncbi.nlm.nih.gov/37543091; https://pubmed.ncbi.nlm.nih.gov/35559682
[5] https://pubmed.ncbi.nlm.nih.gov/39636990
[6] https://pubmed.ncbi.nlm.nih.gov/35642629

diabetes in people ages 8 and older with stage 2 T1D. Key research underlying this approval stemmed from a clinical trial conducted by the NIDDK- and *Special Diabetes Program*-supported Type 1 Diabetes TrialNet, which is a large, collaborative, international consortium designed to perform clinical trials of therapies to delay or prevent type 1 diabetes progression. TrialNet continues to be a unique and critical network for testing novel type 1 diabetes preventive therapies.

While we are excited about teplizumab's success, we also recognize that more research is needed to make type 1 diabetes prevention a reality for more people at risk of developing the disease. As such, TrialNet research continues to shed important light on possible prevention therapies, such as findings from clinical trials that the drug abatacept has beneficial effects on insulin-producing beta cell function but did not delay type 1 diabetes diagnosis, and that the drug hydroxychloroquine did not slow type 1 diabetes progression.[7] TrialNet has one ongoing prevention trial testing a low dose of the immunotherapy drug anti-thymocyte globulin which showed promise in new onset diseases. The Network is also considering testing combinations of drugs with complementary mechanisms of action toward a longer-term goal of permanent prevention.

Understanding the Causes of Type 1 Diabetes

Understanding the causes of type 1 diabetes could also help identify and advance new prevention strategies. We know that a person's risk for developing type 1 diabetes is dependent on both genetic and environmental factors. While research efforts have helped to uncover a large part of the genetic contribution to disease, less is known about environmental contributors to disease.

To identify these environmental components, NIDDK, through the *Special Diabetes Program*, supports an ambitious, long-term clinical research study called TEDDY. TEDDY has screened over 425,000 newborns, enrolling 8,000 who were at high genetic risk of type 1 diabetes. These children are followed until the age of 15 years old, and they and their families have donated over 6 million biological study samples to date.

We have reached a milestone in this study: the youngest TEDDY participant turns 15 this year, marking the end of the participant follow-up phase of the study. I am pleased to report that with this major accomplishment—made possible by decades of dedication by TEDDY researchers and families—final data analyses can begin. In the meantime, TEDDY researchers have already been analyzing vast amounts of data and generating a wealth of knowledge. They are looking at genes, proteins, and metabolites and are also studying the children's microbiomes, viromes, and environmental exposures to understand how these evolve during childhood and how they might influence disease. From this wealth of information, new insights are emerging. For example, analyses of thousands of blood samples from TEDDY children have identified proteins that can predict early stages of the disease, which could lead to improved methods to detect islet autoimmunity before clinical disease onset toward determining who is likely to progress to type 1 diabetes.[8] Continued analyses of TEDDY data and samples—by both

[7] https://pubmed.ncbi.nlm.nih.gov/36920087/; https://pubmed.ncbi.nlm.nih.gov/37708415/
[8] https://pubmed.ncbi.nlm.nih.gov/37390828

TEDDY researchers and the broad scientific community—are expected to continue to yield critical insights that can help us develop new strategies to prevent type 1 diabetes.

Other *Special Diabetes Program*-supported efforts are complementing TEDDY's research to understand factors associated with type 1 diabetes onset. For example, the Type 1 Diabetes in Acute Pancreatitis Consortium is studying type 1 diabetes and other forms of diabetes that occur during or after one or more episodes of acute pancreatitis (sudden inflammation of the pancreas) toward developing new prevention and treatment approaches.

Preventing and Treating Diabetic Complications

People with type 1 diabetes are living longer than ever thanks to new therapies, devices, and other improvements in treatment. Therefore, it is imperative to find ways to prevent and treat the diabetes complications that can arise throughout the lifespan. *Special Diabetes Program*-supported research has already led to tremendous progress, such as research by the National Eye Institute's DRCR Retina Network that have transformed the treatment of diabetic retinopathy and diabetic macular edema worldwide, changing clinical practice guidelines for diabetic eye care.

Through *Special Diabetes Program* support, NIDDK recently began innovative consortia focused on combating type 1 diabetes complications to improve health outcomes, such as research to understand the neurocognitive impact of type 1 diabetes in children to mitigate the risk of developing neurocognitive complications in later life; identify mechanisms that promote the development of cardiovascular disease (CVD) in people with type 1 diabetes to develop CVD prevention and treatment strategies; identify factors that restore awareness of hypoglycemia in adults with type 1 diabetes and impaired awareness of hypoglycemia; and address diabetic foot ulcer healing to prevent amputations. Research is also investigating expanding treatments for people with type 1 diabetes. For example, sodium-glucose cotransporter-2 (SGLT2) inhibitor medications, which are approved to help manage blood glucose levels in type 2 diabetes, have heart-protective effects and may also protect against kidney disease. Determining if these treatments are safe and effective for people with type 1 diabetes could provide a much-needed tool for preventing multiple important complications.

Emerging Opportunities in Type 1 Diabetes Research

The scientific achievements that I described today are just a few examples of recent progress in understanding and combatting type 1 diabetes and its complications. Our efforts were significantly strengthened by the renewal of the *Special Diabetes Program* through the end of Fiscal Year 2025, which enables NIH to continue many of these successful, long-running programs. Sustained, long-term funding of the Program has allowed NIH to launch new clinical trials and support novel research areas. Maximizing the value of the *Special Diabetes Program* and responsibly administering its funds is one of NIDDK's highest priorities.

Throughout the years of SDP support, NIDDK has solicited input from scientific experts, patients, and other interested parties about future research directions in type 1 diabetes and its complications. The most recent workshop for gathering input was held in November 2024 under the auspices of the statutorily required Diabetes Mellitus Interagency Coordinating Committee. Numerous opportunities were identified to build upon the significant progress to date that could lead to new prevention and treatment strategies for people with and at-risk for type 1 diabetes,

such as understanding heterogeneity of type 1 diabetes toward developing more precision medicine approaches; applying AI/ML to type 1 diabetes research, including better prediction models to inform prevention and management; developing cell replacement therapies toward curing the disease; and combating debilitating diabetes complications. NIDDK's support of type 1 diabetes research will continue to be guided by strategic planning efforts and input from scientific conferences and workshops. This input will continue to ensure that NIDDK-supported type 1 diabetes research benefits all Americans who are living with or at risk of developing type 1 diabetes.

Concluding Remarks

I appreciate this chance to share these exciting scientific advances, ongoing efforts, and emerging opportunities in type 1 diabetes research. We are extremely grateful for the continued support of Congress, which provided the recent historic increase in *Special Diabetes Program* funds, that has allowed NIH to vigorously support research to combat type 1 diabetes and its complications. We look forward to continuing our strong partnerships with patient advocacy groups, professional organizations, research institutions, and our sister federal agencies to advance our research objectives. We also thank our dedicated clinical study participants, without whom the clinical research I described today would not be possible. The *Special Diabetes Program* has catalyzed remarkable progress in type 1 diabetes research and has ushered in a new era where individuals with type 1 diabetes have significantly improved health, longevity, and quality of life.

Thank you, Chair Collins, Vice Chair Murray, and Members of the Committee. I will be pleased to answer any questions you have.