Testimony by

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"Maintaining America's Leadership in Biomedical Innovation: FDA's Role in Advancing U.S. Drug Development"

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Good morning, Chairman Guthrie, Ranking Member Pallone, Chairman Griffith, Ranking Member DeGette and members of the Subcommittee.

Thank you for the opportunity to testify before you today. I am honored to be here and greatly appreciate everything this committee and subcommittee have done to help people who live with type 1 diabetes.

I am Dr. Aaron J. Kowalski, Chief Executive Officer of Breakthrough T1D, the leading global type 1 diabetes research and advocacy non-profit organization. Our purpose at Breakthrough T1D, formerly JDRF, is to make everyday life with type 1 diabetes, or T1D, better while driving toward curing the disease. We do this by investing in the most promising research, advocating for progress by working with government to address issues that impact the T1D community, and helping educate and empower people facing this condition.

A scientist by training, I have been fortunate to work for Breakthrough T1D and on behalf of countless individuals and families impacted by T1D for the past 22 years, 7 of which have been in my current role. Like far too many people across the United States and 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.

The focus of today's hearing, FDA's role in advancing innovation, could not be timelier. The progress we are seeing in T1D makes hope for cures realistic. FDA's leadership will be

essential to turning that promise into reality and maintaining U.S. leadership in biomedical innovation.

Type 1 Diabetes – A Costly and Burdensome Disease

First, let me share 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 distinct diseases, they often lead to the same devastating consequences, including heart disease, stroke, kidney failure, and lower-limb amputations. Diabetes overall affects more than 40 million Americans[1], including 2 million with T1D[2], and drives significant costs across the healthcare system. Nearly one in four healthcare dollars and one in three Medicare dollars go toward diabetes care, and diagnosed diabetes cost the United States nearly $413 billion in 2022.[3] T1D drives an outsized share of that burden on a per person basis: Medicare spends an estimated

[1] Centers for Disease Control and Prevention. National Diabetes Statistics Report. Atlanta, GA: U.S. Department of Health and Human Services, Centers for Disease Control and Prevention; 2026.
[2] Smith R, Eisenberg S, Turner-Phifer A, et al. "We Are on the Verge of Breakthrough Cures for Type 1 Diabetes, but Who Are the 2 Million Americans Who Have It?" J Health Econ Outcomes Res. 2024;11(2):145-153. doi:10.36469/001c.124604.
[3] Centers for Disease Control and Prevention, National Diabetes Statistics Report (2026); Parker ED, Lin J, Mahoney T, et al., Economic Costs of Diabetes in the U.S. in 2022, Diabetes Care 47, no. 1 (2024): 26–43. [cdc.gov], [jmcp.org]

$33,787 annually per beneficiary with T1D, more than twice the average Medicare beneficiary.[4]

These realities highlight the importance of continued innovation. New therapies that prevent complications, reduce treatment burden, and/or cure T1D could transform millions of lives while significantly reducing long-term healthcare costs.

Public – Private Partnerships Have Transformed T1D Care

Since our founding in 1970, Breakthrough T1D has invested over $2.5 billion in research. Working alongside federal initiatives like the Special Diabetes Program (SDP), these investments have helped accelerate advances for people living with T1D. The SDP alone has generated an estimated $50 billion in federal healthcare cost savings through T1D research breakthroughs and improved disease management. Thanks to the bipartisan leadership of Representatives DeGette and Bilirakis, and the consistent and overwhelming support of this Committee, SDP-funded research has transformed our understanding of T1D and paved the way for many of the treatments and technologies that are now the standard of care.

Just as importantly, FDA has played a critical role in translating scientific breakthroughs into therapies and technologies that reach patients. FDA-approved innovations such as continuous glucose monitors (CGMs) and automated insulin delivery (AID) systems have

[4] Halhol, Sonia, Michael E. Stokes, Qian Li, Terry Dex, and Laura Wilson. "Healthcare Costs Associated With Type 1 Diabetes in Adults With a Commercial, Medicaid, or Medicare Coverage in US Clinical Practice." Poster RWD18 presented at ISPOR 2024, Atlanta, GA, May 5–8, 2024. https://www.ispor.org/docs/default-source/intl2024/ispor24wilsonrwd18poster136730-pdf.pdf.

improved glucose control, reduced dangerous highs and lows, and eased some of the daily burden of managing T1D. Those innovations reached patients because of sustained research investment and a strong FDA review process.

More recently, FDA approved Tzield, the first and only disease-modifying therapy approved for T1D. Because T1D often progresses silently before symptoms appear, during what we call stage 1 or stage 2 T1D, many people are not diagnosed until they reach stage 3, when deadly symptoms appear, and they require lifelong insulin therapy. Tzield was approved by the FDA in 2022 to delay progression from stage 2 to stage 3 and this year Tzield also received FDA approval for use in stage 3 T1D, becoming the first disease-modifying therapy approved for individuals after diagnosis. It also is the first T1D therapy approved based on preservation of C-peptide, a marker in the blood of how much insulin someone is making.

Tzield's ability to delay the onset of T1D has a tremendous impact on the daily lives of people at risk for and with early onset T1D, their families, and the overall healthcare system. For an extended period it frees them from the constant burden and stress of blood glucose monitoring and insulin administration while giving them the opportunity to learn more about disease management. It enables one to avoid the medical, life-threatening emergency that often accompanies a T1D diagnosis. We commend FDA for following the science and approving Tzield, an approval that is both clinically and emotionally very meaningful. This major milestone, made possible by Congress' investment in the Special Diabetes Program, opens the door to a new generation of disease modifying therapies currently advancing through the clinical development pipeline.

Significant Unmet Needs

Yet despite this extraordinary progress, T1D remains a serious, lifelong autoimmune disease with no cure. Managing it requires around-the-clock attention to insulin dosing, glucose levels, food, exercise, and the risk of life-threatening complications. There are no breaks or days off.

As documented in Breakthrough T1D's article "The Urgent Need for Breakthrough Therapies and a World Without Type 1 Diabetes," published in Diabetes Therapy, people with T1D continue to face significant health burdens and a shorter life expectancy despite advances in treatment.[5] This underscores the need for sustained investment in research and innovation to deliver better treatments and, ultimately, cures.

FDA must keep up with the science; opportunities & recommendations

Alongside the significant unmet needs of T1D, there is enormous hope. T1D science is advancing at an extraordinary pace, as are the regulatory science tools that are used to evaluate these breakthroughs. We are grateful for FDA's partnership in advancing innovations for people living with T1D, and as science evolves, FDA must continue to modernize so patients can benefit from new discoveries safely and effectively.

One area where the United States has fallen behind is deceased donor islet transplantation. While countries including China, France, Canada, the United Kingdom, Australia, and Japan have provided access to this therapy through whole organ

[5] Starr, L., Dutta, S., Danne, T. *et al.* The Urgent Need for Breakthrough Therapies and a World Without Type 1 Diabetes. *Diabetes Ther* **16**, 1063–1076 (2025). https://doi.org/10.1007/s13300-025-01735-6

transplantation frameworks, an outdated U.S. regulatory approach continues to limit patient access to these transplants. The Department of Health and Human Services has the authority to modernize this framework by regulating deceased donor islets consistently with other donated organs, preserving rigorous safety standards while expanding access for use by researchers and for eligible individuals with T1D. It should do so. Thank you, Representatives DeGette and Bilirakis, for recognizing this important issue and sending a letter to FDA asking them to reclassify deceased donor islets.

Looking ahead, for the first time, we can realistically envision restoring insulin-producing cells and fundamentally changing the course of T1D. For those affected by this disease, this is not just a scientific milestone; it is real hope. Potentially curative therapies are advancing through late-stage development, with multiple trial participants coming off insulin entirely and a pivotal trial is now underway in the United States. Whether these breakthroughs reach patients quickly, and are developed and manufactured in the United States, will depend in large part on FDA's ability to keep pace with the science. The U.S. has a genuine opportunity to become the first country to approve and manufacture a scalable curative therapy for T1D. Let me repeat that: The U.S. has a genuine opportunity to become the first country to approve and manufacture a scalable curative therapy for T1D.

FDA has done this before. Its leadership in establishing a regulatory pathway for automated insulin delivery systems helped foster a thriving marketplace of dozens of innovative products that have transformed diabetes management. We now need that same FDA leadership for cell therapies.

Breakthrough T1D has stepped up to help. Last year we published a roadmap outlining how cell therapies should evolve, from today's deceased donor islets, which require broad immunosuppression, to manufactured islets that require reduced or no immunosuppression and are available to more people with T1D.[6] Realizing that vision will require innovation not only in the therapies themselves, but also in clinical trial design, manufacturing standards, and preclinical trial requirements. To support this work, Breakthrough T1D and external experts published a second roadmap identifying ways to advance these therapies toward clinical trials, regulatory review, and eventual approval safely and efficiently.[7]

Last December I visited China and saw firsthand the scale of investment in T1D and cell therapy research. While the United States remains the global leader in biomedical innovation, that leadership is not guaranteed. Maintaining it will require a strong, scientifically capable FDA that can support the rapid development and review of breakthrough therapies.

To help deliver the next generation of therapies and cures for T1D and sustain America's leadership in biomedical innovation, FDA should focus on five priorities:

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[6] Marjana Marinac et al., "Future Directions and Clinical Trial Considerations for Novel Islet β-Cell Replacement Therapies for Type 1 Diabetes," Diabetes 74, no. 9 (2025): 1452–1463, available at https://doi.org/10.2337/dbi24-0037.

[7] Chengyuan Press et al., "Allogeneic Islet Products for Type 1 Diabetes: Navigating Nonclinical and Manufacturing Regulatory Expectations," Stem Cell Reports (July 6, 2026), available at https://doi.org/10.1016/j.stemcr.2026.102998.

1. Provide clear and consistent regulatory expectations across the agency.

Medical product developers need clear and consistent expectations from FDA. When requirements are unclear or inconsistent, the resulting uncertainty increases risk, cost, and development timelines. This discourages investment in U.S. innovation and can drive sponsors to pursue development elsewhere. FDA's policies should be grounded in sound science and applied consistently across the agency, regardless of the disease area or review center involved.

2. Engage proactively with researchers, patient organizations, and product developers.

FDA's willingness and ability to engage with researchers, patient organizations, and developers directly affects the pace of innovation. Similar to other diseases, T1D is entering a new era of cell therapies and disease-modifying therapies that have the potential to delay, prevent, and even cure the disease. As science and the development of these therapies advance, FDA must remain an active participant in the scientific community, helping translate emerging evidence into efficient regulatory pathways that accelerate patient access to breakthrough therapies. FDA's gold standard can and should be applied in the least burdensome way.

3. Modernize clinical trial requirements and accelerate qualification of novel endpoints and biomarkers.

Clinical trial requirements developed for insulin therapies are not always a good fit for treatments aimed at preserving insulin-producing cells, slowing the progression of T1D, or

potentially curing the disease. Several promising therapies have been discontinued despite encouraging clinical results because developers viewed regulatory requirements as too burdensome or insufficiently aligned with science. When that happens, patients lose access to potentially life-changing innovations.

FDA's recent accelerated approval of Tzield for stage 3 T1D marked an important step forward. For the first time, a regulatory decision in T1D relied on C-peptide, a measure of the body's insulin production. For decades, despite broad scientific consensus on its value[8], FDA was reluctant to rely on C-peptide for regulatory decision-making. This milestone demonstrates that modern regulatory approaches can accelerate innovation while maintaining rigorous standards for safety and effectiveness.

While this progress is encouraging, FDA's broader framework for evaluating C-peptide has not fully caught up with the science.[9] Continued modernization will be essential to ensuring that innovative therapies are developed and approved in the United States.

Patients cannot afford to wait another generation for regulatory frameworks to catch up with scientific advances. FDA should strengthen and modernize its endpoint and biomarker qualification programs so that promising therapies can reach patients more quickly. In particular, the Biomarker Qualification Program established through the 21st Century Cures Act, led by Ranking Member DeGette and former Chairman Fred Upton, has

[8] Palmer JP, Fleming GA, Greenbaum CJ, et al. "C-peptide Is the Appropriate Outcome Measure for Type 1 Diabetes Clinical Trials to Preserve Beta-Cell Function: Report of an ADA Workshop, 21–22 October 2001." Diabetes. 2004;53(1):250-264.
[9] Herold KC, Greenbaum CJ, Beam CA, et al. "Evidence for C-Peptide as a Validated Surrogate to Predict Clinical Benefits in Trials of Disease-Modifying Therapies for Type 1 Diabetes." Diabetes Care. 2024;47(4):686-696. PMID: 38349844.

not consistently provided the clarity, predictability, and efficiency needed to advance innovation. Congress should work with FDA, researchers, developers, and patient advocates to ensure the program fulfills its intended purpose.

FDA can also learn from international regulatory models. The European Medicines Agency's Qualification of Novel Methodologies program provides a structured framework for evaluating innovative tools and approaches. Similar reforms here could improve clarity, predictability, and scientific flexibility while maintaining FDA's gold standard for safety and effectiveness.

4. Fully incorporate the patient perspective into benefit-risk assessments.

FDA decisions should reflect the experiences and priorities of the people living with the disease. When the burdens and risks faced by patients are not fully incorporated into regulatory decision-making, clinical development becomes more restricted, therapies aren't evaluated on their ability to improve lives, and we shift towards an overly conservative, slow, risk-averse approach that pushes companies to pursue trials overseas. The patient's voice should remain central to FDA's evaluation of benefits, risks, and the meaningfulness of a therapy to the people who need it. Breakthrough T1D is leading an effort to rigorously capture and quantify how people with T1D balance the risks and benefits of novel and curative therapies. We are currently analyzing these data and look forward to working with FDA to ensure these important findings help inform regulatory decision-making.

5. Maintain a strong and stable workforce.

Finally, none of these goals can be achieved without a strong FDA workforce. Workforce instability, loss of institutional knowledge and expertise, and resource constraints can delay product reviews and slow patient access to innovation. I want to commend FDA career professionals we work with who remain deeply committed to the Agency's mission, to helping improve people's lives and to maintaining a highly skilled scientific workforce at FDA. They are critical to progress.

While I've focused my remarks on T1D, these challenges are not unique to our community. Patients across many disease areas would benefit from more predictable regulation, modern clinical trial frameworks, stronger incorporation of patient perspectives, and a well-resourced FDA. By addressing these systemic challenges, Congress and the Agency can strengthen the entire biomedical innovation ecosystem and ensure the United States remains the world's leader in developing and delivering life-changing therapies.

There are meaningful actions now matching the encouraging words from FDA to bring urgency, efficiency, and patient-focused reforms to reviewing new therapies. We are encouraged by HHS's new Operation TrialBlazer initiative. Efforts to accelerate development timelines and reduce unnecessary regulatory burdens, while maintaining rigorous standards for patient safety, could help bring promising therapies to patients sooner. We welcome the opportunity to engage further as this initiative moves forward.

The story of type 1 diabetes over the past 25 years is a story of what happens when Congress invests in research, FDA embraces science, and patients remain at the center of innovation. We now stand at the threshold of therapies that can delay, prevent, and cure

T1D, and the science driving this progress is already informing research into other autoimmune and metabolic diseases. On behalf of the millions of families, researchers, clinicians, and patients depending on this work, we stand ready to work with Congress and with FDA leadership to deliver on that promise. Those living with T1D have waited long enough.