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March 6, 2026

The Honorable Robert F. Kennedy, Jr.
Secretary
U.S. Department of Health and Human Services
200 Independence Avenue, S.W.
Washington, DC 20201

Re: Proposal to Reclassify Deceased Donor Islet Cells as Organs for Transplantation Purposes

Dear Secretary Kennedy:

I write to share exciting news and to seek your partnership. There are 1.6 million Americans living with type 1 diabetes (T1D) and we find ourselves on the cusp of transformative and even curative treatments for some patients who are eligible and amenable to islet cell transplantation. Just as our community is collaborating with the administration on equally meaningful regulatory modernizations for clinical trials using the endpoint c-peptide, we must also partner in response to recent clinical progress on deceased donor islet cell transplantation.

Outdated regulatory classifications are limiting access to this safe, effective, and potentially curative therapy for those who are eligible. We urge the Department of Health and Human Services (HHS) to take administrative action and seize the opportunity to lead a targeted regulatory reclassification that would expand access for eligible patients while maintaining rigorous safety oversight. Specifically, we believe that with appropriate quality and safety controls, unmodified deceased donor islet cells should be reclassified as organs for purposes of transplantation from their current classification as biological products requiring approval of a Biologics License Application (BLA).

As the leading global T1D research and advocacy organization, Breakthrough T1D's mission is to accelerate life-changing breakthroughs to cure, prevent, and better treat T1D and its complications. We do this by investing in the most promising research, advocating for progress by working with the federal government to address issues that impact the T1D community, and helping educate and empower individuals facing this chronic, autoimmune condition which requires a person to depend on insulin to live.

Since our founding in 1970, we have committed over $2.5 billion in research grants. This private investment works hand-in-hand with federal initiatives like the Special Diabetes Program (SDP) that has delivered an extraordinary return on investment through life-

changing breakthroughs in disease management.[1] The SDP, in particular, has proven that the federal government is key to achieving cures for T1D. Building on this strong public-private partnership, we believe HHS could lead a targeted regulatory realignment of deceased donor islet cells to improve availability of this life-changing therapy in the United States. We are seeing a rapid acceleration toward progress in other countries and are committed to working with you to deliver the same innovation and access to people with T1D in the U.S.

In parallel with regulatory realignment, we urge you to lead efforts to tackle barriers to coverage under federal health programs (and set a clear expectation for commercial insurers as you so effectively did on prior authorization reform) so that eligible people living with T1D can access deceased donor islet cell transplants in practice, not just in theory.

Attached for your consideration is additional background and a proposed framework to meet this achievable goal. The research we have funded, coupled with input from leading clinicians, scientists and foundations contributing to diabetes cure-focused research over the decades, make an extraordinarily compelling case for action.

As CEO of Breakthrough T1D and someone who has lived with the disease for over 40 years, I stand ready to engage with HHS to use your existing authority to advance policies that accelerate safe access, strengthen US leadership in innovation, and move us closer to curing T1D. We look forward to working with you and your team to seize this timely opportunity for federal leadership to align regulation and coverage so that a proven, life-changing therapy can reach eligible Americans living with T1D. Thank you for your consideration.

Respectfully,

Aaron J. Kowalski, PhD

CEO, Breakthrough T1D

[1] https://advisory.avalerehealth.com/wp-content/uploads/2025/07/20250925-Breakthrough-T1D-SDP-White-Paper_vPUB.pdf.

Reclassification Proposal for Deceased Donor Islet Cell Transplantation

Recent clinical trial results have sparked a growing view within the type 1 diabetes (T1D) community – voiced by those with T1D, their families and health care providers – that there is an urgent need to exercise current authority and consider a regulatory reclassification to increase availability of deceased donor islet cell transplantation.

Background on Deceased Donor Islet Cell Transplantation

Deceased donor islet cell transplantation was discovered over forty years ago and a National Institutes of Health (NIH)-supported Phase III clinical trial demonstrated the safety and efficacy of the procedure over a decade ago for people with T1D who experience hypoglycemia unawareness and severe hypoglycemia. Continued research funded by Breakthrough T1D and others has shown that deceased donor islet cell therapies remain a key component of curing T1D.

In 2023, the Food and Drug Administration (FDA) approved a deceased donor islet cell product, Lantidra (donislecel-jujn), and yet, since that time, only one use of that product has been publicly reported. In our view, this lack of uptake indicates that the current regulatory pathway in the U.S. is not supporting meaningful patient access to this safe and effective therapy.

Current U.S. Regulatory Framework

Currently, the FDA regulates deceased donor islet cells as biological products requiring approval of a Biologics License Application (BLA). However, for hospitals and medical centers, the cost and resource demands of filing a BLA or qualifying an additional facility under an approved BLA are often prohibitively burdensome. More significantly, this regulatory approach is a misfit for deceased donor islet cells, which are more biologically analogous to whole organs rather than manufactured biologic products.

An administrative solution is both appropriate and feasible. The current U.S. regulatory approach to deceased donor islet cells diverges from how whole solid organ transplants (such as pancreas transplants) are handled under U.S. law. Further, the current U.S. regulatory approach is outdated relative to how deceased donor islet cells are regulated in many other developed nations, including France, Canada, the United Kingdom, Australia, China, and Japan. There are reports that these countries collectively perform hundreds of deceased donor islet cell transplants each year under organ-based oversight.

Rationale for Proposed Regulatory Reclassification

To help ensure the availability of safe deceased donor islet cells and expand availability of such products to eligible adults with T1D in the U.S., we believe that the regulatory definition of organs should be expanded to include unmodified deceased donor islet cells under oversight of the Organ Procurement and Transplantation Network (OPTN) while maintaining FDA oversight of manufactured cell therapies and deceased donor islet cell therapies that undergo any further modification. Importantly, this position is supported by many leading transplant clinicians and scientists from renowned academic institutions across the U.S.

Under the National Organ Transplant Act (NOTA), 42 U.S.C. § 274, the Secretary of HHS oversees the transplant of human organs through the Organ Procurement and Transplantation Network (OPTN). For more than four decades, NOTA has provided a framework for the nation's organ donation and transplantation system, supporting oversight through the OPTN. Since then, the U.S. now performs organ transplantation at an unprecedented scale—48,149 organ transplants in 2024 and ~915 pancreas transplants in 2023.[2]

A clear and important scientific distinction supports such consideration. Deceased donor islet cells that do not undergo any further modification and are transplanted into the liver are micro-organs and are biologically analogous to transplanted whole organs such as pancreata, kidneys, livers, and hearts. Experience has shown that applying pharmaceutical manufacturing standards to unmodified deceased donor islet cells imposes expectations of drug uniformity on inherent biological variability and impedes access. In contrast, we believe that manufactured islet cells, as well as deceased donor islet cells that involve further modification, should remain subject to FDA's BLA oversight.

The U.S. can draw from the experience of other countries performing these procedures at designated or accredited centers as well as the experience from the clinical trials performed in the U.S. to develop appropriate qualification and quality standards for deceased donor islet cell transplantation under NOTA in a manner that preserves patient safety while expanding availability.

Specifically, we recommend consideration of the following measures, which are informed by approaches adopted in other countries:

- **Accredited Centers of Excellence:** Islet isolation and/or transplantation will be permitted only in UNOS/OPTN accredited centers that ensure safety by meeting well-defined standards of qualification, infrastructure and appropriately trained and experienced personnel.
- **Stringent Quality Controls:** Implementation of rigorous measures for deceased donor islet cell purification and potency.
- **Distribution Framework:** A formalized plan for deceased donor islet cell distribution to the point of care.
- **Scalability:** A strategic roadmap for expanding transplantation capabilities to additional centers.

This approach promises greater availability of deceased donor islet cells as a safe and effective therapy for eligible adults living with T1D. Moreover, taking this action would:

- Preserve FDA authority where appropriate (e.g., for manufactured cell therapy products and deceased donor islet cell products that involve further modification).

[2] Health Resources & Services Administration (HRSA), Organ Transplants Exceeded 48,000 in 2024; A 3.3 Percent Increase from the Transplants Performed in 2023 (Jan. 15, 2025); HRSA, OPTN/SRTR 2023 Annual Data Report: Pancreas.

- Align regulation with biological reality by treating micro-organs, such as unmodified deceased donor islet cells, as organs.
- Enable sustainable academic innovation within the U.S. research community.
- Strengthen American competitiveness on the global path to T1D cures.

Conclusion

Deceased donor islet cell transplantation is a safe, effective, and scientifically validated therapy that remains largely inaccessible in the United States due to an outdated regulatory classification that no longer reflects biological reality or global clinical practice. The Secretary of HHS has clear authority under existing law to modernize oversight by reclassifying unmodified deceased donor islet cells as organs under the Organ Procurement and Transplantation Network, while preserving FDA oversight where appropriate. Doing so would expand access for eligible adults living with T1D, support sustainable academic innovation, and align U.S. policy with international best practice—without compromising patient safety. Breakthrough T1D stands ready to partner with HHS to implement this targeted, feasible solution and ensure that proven science translates into meaningful, life-changing care for Americans with T1D.