Aaron J. Kowalski, Ph.D.

CEO, Breakthrough T1D

House Energy & Commerce Health Subcommittee Hearing

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

July 15, 2026

Type 1 diabetes (T1D) is a lifelong autoimmune disease that affects approximately 2 million Americans and requires constant management with insulin. Despite significant advances in treatment, people with T1D continue to face severe health risks, a substantial daily burden, and reduced life expectancy.

Today, T1D research is entering a new era. Disease-modifying therapies and cell-based therapies now offer the realistic possibility of delaying, preventing, or even curing T1D. Multiple cell therapy programs have enabled some clinical trial participants to come off insulin entirely, and pivotal trials are underway in the United States. The United States has a genuine opportunity to become the first country to approve and manufacture a scalable curative therapy for T1D.

Realizing that opportunity will require FDA to modernize alongside the science. As Congress begins work on the next PDFUA reauthorization, Breakthrough T1D recommends five FDA-related priorities to strengthen innovation and accelerate patient access to breakthrough therapies:

1. **Provide clear and consistent regulatory expectations** to reduce uncertainty, encourage investment, and keep innovation in the United States.
2. **Engage proactively with researchers, patient organizations, and developers** to translate emerging science into efficient regulatory pathways.
3. **Modernize clinical trial requirements and accelerate qualification of biomarkers and endpoints.** FDA should build on recent progress, including use of C-peptide in regulatory decision-making, and strengthen the Biomarker Qualification Program established by the 21st Century Cures Act.
4. **More fully incorporate patient perspectives into benefit-risk assessments.** Patients understand the burden of their disease and should help inform evaluations of innovative and potentially curative therapies.
5. **Maintain a strong and stable FDA workforce.** Scientific expertise, institutional knowledge, and adequate resources are essential to efficient review of breakthrough therapies.

Maintaining FDA's gold standard for safety and effectiveness is essential. However, patients cannot afford to wait for regulatory frameworks to catch up with science. By modernizing regulatory pathways while preserving rigorous standards, Congress and FDA can accelerate patient access to breakthrough therapies and strengthen America's leadership in biomedical innovation.

The story of T1D over the past 25 years shows what is possible when science, government, and patient advocacy work together. We now stand at the threshold of therapies that can delay, prevent, and potentially cure T1D. People living with T1D have waited long enough.