PES Leadership Needed: Protecting Standards of Care for Teplizumab Infusions in Type 1 Diabetes
A unified statement from U.S. pediatric endocrinology leaders

Dear PES Leadership,

Following Sanofi's recent announcement that teplizumab has been accepted for expedited review in the United States for stage 3 type 1 diabetes through the FDA Commissioner's National Priority Voucher Pilot Program, we are writing to express the critical need to include pediatric endocrinologists with direct clinical and research experience administering teplizumab in discussions about how this therapy will be implemented nationally, and likely globally, across both stage 2 and stage 3 of type 1 diabetes.

The potential approval of an immunotherapy to delay the loss of residual beta cell function in stage 3 type 1 diabetes is a remarkable milestone for our field, but also a moment of vulnerability. From our extensive real-world experience delivering teplizumab infusions for stage 2 T1D, we know that the greatest obstacles are staffing, space constraints (particularly weekend coverage over the 14-day course), and limited provider familiarity with immunotherapy monitoring. These challenges are significant but solvable with strong institutional support, collaboration, and advocacy.

Why Clinical Oversight Matters

There is growing concern that home infusion models may be promoted to accelerate access. Many colleagues without experience giving teplizumab but seeking access for their patients may initially accept these models without recognizing their implications for patient safety, the future of immunotherapy in type 1 diabetes, and the long-term stability of our specialty.

While well-intentioned, home administration risks delayed recognition of infusion reactions, inconsistent monitoring, and fragmented follow-up, ultimately compromising outcomes and undermining the central role of diabetes specialists. Once a home-based model is approved for use, it becomes extremely difficult to reverse, making it essential that patients begin treatment in the safest setting from the outset. Because prior authorizations are tied to a specific infusion site, the initial approval effectively locks in the location of care or at the very least requires providers to obtain two separate authorizations, one for infusion center and one for home infusion. This process greatly limits flexibility if safety concerns arise or patient needs evolve. Moreover, adverse effects and glycemic responses to teplizumab are variable, particularly in newly diagnosed patients adjusting to insulin and glucose fluctuations, requiring close, continuous clinical observation.

Without coordinated planning among endocrinology teams, infusion services, and hospital or institutional administrators, providers may be expected to oversee large numbers of infusions without appropriate reimbursement or protected resources. Pediatric endocrinologists leading the care of patients with stage 3 type 1 diabetes must be supported with adequate staffing, infrastructure, and billing mechanisms to sustain this high-quality care during and after infusion therapy.

Reinvestment of drug-acquisition margins and infusion-administration revenue into the endocrine departments providing clinical oversight is essential. Without such reinvestment, our specialty will continue to erode, further limiting our ability to recruit and retain future trainees in pediatric endocrinology. Pediatric endocrinology remains one of the lowest-compensated medical specialties despite the complexity and breadth of care provided. Teplizumab and future immunotherapies present an opportunity to align institutional reimbursement with the clinical value and subspecialty expertise pediatric endocrinologists provide, ensuring the sustainability of our field while improving patient outcomes.

Lessons from Other Pediatric Specialties

Other pediatric fields have already set clear standards:

- NASPGHAN recommends that any non-hospital biologic infusion for children ≤ 12 years be staffed by pediatric-trained, PALS-certified nurses, and that hospital-based administration remain preferred (Barfield et al., J Pediatr Gastroenterol Nutr 2018).
- Rheumatology limits home infusions to exceptional cases, requiring staff trained in pediatric emergency management (Wahezi et al., Arthritis Rheumatol 2020).

These principles - safety, supervision, pediatric expertise, and rapid response - apply directly to disease-modifying therapies for autoimmune diabetes, where patients are acutely adjusting to insulin titration, glucose monitoring, and psychosocial adaptation to a lifelong condition.

Infrastructure, Safety, and the Value of Clinical Care

A major concern is that existing infusion infrastructure at most centers cannot accommodate the expected increase in patient volume following approval. The solution cannot be to lower safety standards by shifting to home infusion. Instead, we must advocate for institutional investment so that diabetes teams can deliver therapy seven days per week, using hospital-trained or endocrine-trained infusion nurses with joint expertise in immunotherapy and diabetes management. Infusions should occur in settings that are integrated within the patient's diabetes or endocrine care program, whether hospital-based or within a dedicated specialty clinic, allowing immediate coordination between the infusion team, diabetes educators, and prescribing endocrinologists. This approach provides novel opportunities to maximize diabetes education for new-onset patients and ensures continuity of care.

Care delivered in a specialized outpatient or hospital-based setting ensures:

- Immediate access to laboratory testing and rapid turnaround, enabling same-day clinical decisions.
- Direct, hands-on clinical assessment by trained staff who can distinguish "sick vs. not sick" and escalate care appropriately.
- Real-time counseling and patient education, which decreases after-hours calls, improves adherence, and strengthens long-term relationships.

These in-person interactions provide irreplaceable clinical value and improve patient experience, but they require protected time, training, and billing structures that compensate for the expertise provided. Advocating that revenue from drug administration and related clinical care be reinvested into endocrine departments is essential to maintain trained personnel, support infrastructure upgrades, and sustain our specialty long-term.

While hybrid approaches, such as completing a portion of the 14-day course at home after completing the first 7 days in a hospital or specialty outpatient setting, may present a reasonable way to alleviate some of the expected infusion chair shortages and optimize patient convenience, home-based treatment should remain the exception. If ever considered (for older, stable adolescents or adults), it should only occur under direct supervision by hospital- or endocrinology-trained personnel, not general home-health contractors, given documentation of variability and safety concerns (Baker et al., 2021; Barfield et al., 2018; Cox et al., 2013).

Call to Action

Given the urgency of this issue, we urge PES to expedite the development of guidance defining standards for safe, clinically supervised delivery of disease-modifying therapies in type 1 diabetes. This is time-sensitive: the National Priority Voucher pilot shortens FDA review timelines from 10–12 months to as little as 1–2 months.

We understand that developing a full position statement takes time. Therefore, we recommend that PES post this letter publicly on its website as an immediate Society Letter of Concern and Guidance. This would allow programs to reference a unified pediatric endocrinology stance while formal recommendations are being developed.

Key priorities should include:

- Affirming that infusions must occur under trained medical supervision in accredited clinical settings;
- Encouraging institutional investment in infusion access solutions (shared staffing, extended hours, weekend coverage);
- Ensuring that revenue from drug acquisition and infusion administration is reinvested into endocrine departments to sustain specialized staff, training, and infrastructure;
- Reinforcing the central role of diabetes specialists and multidisciplinary teams in managing early disease and supporting families through diagnosis and immunotherapy.

This letter represents a unified call from pediatric endocrinology leaders at centers that deliver gold-standard diabetes care and have administered teplizumab or similar agents in both research and early clinical settings.

Together, we must act now, not to resist change, but to shape it, so that this new era of disease-modifying therapy strengthens our specialty, protects our patients, and ensures that future generations of pediatric endocrinologists can continue to deliver the highest standard of care.

With appreciation for your leadership and partnership,

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