CAR T Cell Therapy Long-Term Follow-Up Studies — FDA-Requested Listening Session

June 23, 2026, 1.5 hours

Objectives of Session

This Patient Listening Session was requested by CBER/OTP/OCE/Division of Clinical Evaluation Hematology. Patients who receive CAR T cell therapy require 15 years of follow-up to monitor for delayed adverse effects, including the risk of secondary malignancies and neurological toxicities. The Division wanted to better understand: the burden, barriers, and challenges of adhering to long-term follow-up (LTFU) studies after receiving an FDA-approved CAR T cell therapy; any unique burdens, barriers, and challenges for pediatric and young adult patients, including those attending school away from home who return only during holidays or semester breaks; and, in the rare event a patient develops a new cancer (secondary malignancy) after CAR T cell therapy, the burdens, barriers, and challenges of receiving biopsies and appropriate testing for their tumor specimen.

Discussions in FDA Listening Sessions are informal and not meant to replace, but rather complement, existing patient engagement opportunities in the Agency. All opinions, recommendations, and proposals are unofficial and nonbinding on FDA and all other participants. This report summarizes the input provided by persons from the CAR T cell therapy community at the meeting. To the extent possible, the terms used in this summary to describe the health needs, perspectives, preferences, and impacts reflect those of the individual participants. This report is not meant to be representative of the views and experiences of the entire CAR T cell therapy patient population or any specific group of individuals or entities. There may be experiences that are not mentioned in this report.

Participants Represented

- **Number of Patient Participants:** Five
- **Number of Caregiver Participants**: One
- **Sex:** The patients and caregiver participant represented their loved ones, who were three females and three males.
- **Geographical location*:**
    - One participant was from the Southern United States (U.S.).
    - Five participants were from the Northeastern U.S.

***Please click here for a map of the geographic regions referenced**

- **Ages:** The patients and caregiver participants represented CAR T cell therapy recipients ranging in age from 19 to 68 years old.

Summary of Discussion by Question

Round 1: Background and Treatment Experience

1. Can you briefly tell us about your CAR T cell therapy experience? How long ago did you receive your infusion? Are you still receiving follow-up care at the same center where you were treated?

- All seven participants/caregivers reported that they (or their child) are still receiving follow-up care at the same center where they were originally treated or are in the process of a planned transition (e.g., from a pediatric to an adult treatment center).
- Time since infusion varied widely among participants, ranging from approximately 1 year to many years post-treatment.

2. What were you told about long-term follow-up (LTFU) when you or your child received CAR T cell therapy? How does that compare to your experience?

- Most participants reported receiving both verbal and written information about LTFU at the time of treatment and generally described this information as accurate or better than expected.
- One participant reported receiving no written information from their treatment center and instead read the FDA Risk Evaluation and Mitigation Strategy (REMS) document independently.
- One participant described their actual CAR-T experience as substantially more difficult than expected, including rare side effects that required extended hospitalization and multiple ICU stays.

Round 2: The LTFU Experience – Visits, Tests and Logistics

3. How would you describe your understanding of the purpose of LTFU? What do you believe it is for, and where does that understanding come from?

- Participants generally understood LTFU to serve two main purposes: monitoring for known or expected long-term risks (such as secondary cancers or immune-related issues), and identifying new or unexpected side effects not observed during clinical trials.
- Several participants noted that the volume of information provided around the time of treatment made it difficult to focus on or fully process specific long-term risks, describing that period as chaotic.
- One participant, treated shortly after a particular CAR-T product's approval, noted that some risks now known were not discussed with her, since they were not yet known at the time of her treatment.

4. How were you informed about the long-term side effects of CAR T cell therapy, including the risk of developing a new cancer? Was this information provided verbally or in written form? Would written materials have helped you better recall information about side effects?

- Limited direct feedback was captured for this question; one participant indicated that written materials helped them better focus on and recall information about potential side effects.

Round 3: Burdens and Barriers to Continuing LTFU

5. Can you describe what a typical LTFU appointment looks like for you? How have LTFU visits changed over time? In what ways, if any, do you find the frequency or type of testing to be burdensome or excessive?

- Participants described a general pattern of LTFU visits decreasing in frequency and intensity over time — from frequent, intensive testing (e.g., bone marrow biopsies, PET scans, weekly or monthly labs) in the months immediately following treatment, to periodic in-person or telehealth visits (ranging from monthly to annual) in later years.
- Several participants said their visit frequency had decreased to every 3-6 months or annually with some visits through telehealth, and that some services, such as immunoglobulin infusions, could be handled at a local oncology practice.
- Most participants did not describe the frequency or type of testing at their current stage of follow-up as overly burdensome, though several described the earlier, more intensive testing period as difficult.

6. Can you describe the logistical challenges of getting to and attending your LTFU appointments? What financial impacts have you experienced because of LTFU?

- Travel time to appointments varied widely, from a few minutes to several hours each way; participants cited traffic, distance, and cost of living near treatment centers as burdens.
- Financial impacts mentioned included travel costs (gas, parking), lost wages or job loss related to disability leave related to the complications of CAR T cell therapy, and the burden on caregivers who had to travel to and stay near the treatment center around the time of CAR T cell treatment.
- One participant described the early post-treatment period as especially difficult due to a lack of a support system at the time.
- One participant, a college student, described difficulty finding time to miss school for travel to LTFU care located far from her college.

Round 4: Coordination with Local and Community Providers

7. What has been the most difficult aspect of long-term follow-up for you as a patient or caregiver? Can you describe any circumstances in which you missed, delayed, or thought about stopping your follow-up visits?

- Multiple participants described the psychological or emotional burden of LTFU as more difficult than the logistical burden, including anxiety while awaiting test results (sometimes referred to as “scanxiety”) and the emotional toll of periodic reminders of their cancer history.
- No participant reported having stopped or seriously considered stopping their follow-up visits altogether, though rescheduling was common; one caregiver noted her adult daughter has expressed uncertainty about continuing visits once she reaches longer remission milestones.

- One participant described relapsing during a period when clinical trial data suggested relapse was unlikely, which they described as the most difficult and devastating part of their LTFU experience.

8. How does the travel/frequency/length of the visit affect your work/school/family/personal life? How has the experience of participating in LTFU changed as you have gotten older, or when away from home for college, an internship, or work?

- Participants described a range of impacts on work, including needing to take full days off for appointments, difficulty finding employment that accommodates a demanding visit schedule following CAR T cell treatment, and a preference for remote work due to both scheduling and immunocompromised status.
- Parents/caregivers of young children described additional logistical burden related to childcare and minimizing young children's exposure to radiation or infection risk after treatment.
- Participants who received treatment as children or young adults described impacts on school, including missed school days, social and academic effects from missing a full academic year as a result of treatment and follow up visits, and difficulty maintaining LTFU care while away at college.
- Being immunocompromised was described by multiple participants as limiting social activities (e.g., avoiding large gatherings, concerts, or crowds) well beyond the active treatment period.

Round 5: Risk of New T cell cancer and CAR T Specific Testing

9. Can you describe your experience coordinating care between your CAR T treating center and a local oncologist or community provider? What worked well and what felt fragmented or unclear?

- Limited direct feedback was captured for this question; one participant noted that quality of care is not uniform across locations and described relocating to access better care at a tertiary CAR T treatment center.
- One caregiver described strong trust in her daughter's original treatment team and reluctance to shift any portion of care to a local primary care provider, citing concerns about whether a non-specialist would have the necessary expertise or complete medical history.

10. If you were to develop a new symptom or an abnormal test result, how would you go about deciding what to do and who to contact first?

- No responses were captured for this question.

Round 6: Improving LTFU – Patient-Centered Solutions

11. What information about your LTFU visits is communicated back to you, and how? What was your understanding of why a biopsy or blood sample might need to be sent to the CAR T manufacturer or a specialized laboratory?

- Most participants described using an online patient portal (e.g., MyChart) to receive test results, generally within about 24 hours, and described this as efficient.

- Participants generally expressed understanding of, and openness to, the possibility that a biopsy or blood sample might be sent to the CAR T manufacturer or a specialized lab if a new cancer were to develop, though for some this understanding was limited at the time of treatment due to the urgency of starting therapy.
- Several participants expressed reservations about whether a local or community hospital would be equipped to properly handle, process, or ship a biopsy sample for CAR T-specific testing, with at least one describing a prior negative experience with a poorly performed biopsy at a local facility.

Additional Questions (if time allows)

12. What changes to long-term follow-up would make the greatest difference in reducing burden for patients, parents, and caregivers? What role could technology play?

- Participants generally supported the use of digital tools such as patient portals, apps, or online reporting tools to help manage LTFU, though several cautioned that older patients and those in rural areas may be less comfortable with mobile apps and may be better served by computer-based tools.
- Most participants were reluctant to discontinue LTFU as they saw this as an obligation to maintain their wellness. Most participants did state that as the frequency of follow-up decreased, it posed less of a burden to them.
- Virtual visits and access to local laboratories located near home may help reduce the burden of long-term follow-up (LTFU).
- Participants who transitioned from pediatric to adult care described this as an emotionally difficult transition, though those who had already been through it described it as ultimately manageable and, in some cases, positive due to continuity of relationships with their care team.
- One participant highlighted the burden of fragmented electronic health records when transitioning between health systems and suggested improved interoperability between patient portal systems.

13. Follow-up: Between 10-15 years, how do you think your compliance with continuing follow-up would be? How do you stay engaged?

- Participants generally expressed strong intent to remain engaged with LTFU over the long term (10-15 years), citing peace of mind as a primary motivator.
- One participant raised concern that institutions themselves may be more likely than patients to let follow-up lapse once a patient has been in remission for an extended period (e.g., 5 years), suggesting that patients may need to proactively advocate to continue LTFU.
- One participant described channeling his experience into advocacy, working with his treatment center to help expand access to immunotherapies for other pediatric patients.
- One participant noted that the ability to complete follow-up visits at a hospital or facility near their home would facilitate compliance with LTFU.

14. Follow-up: In any way, did having to do follow-up restrict your ability to go to colleges further away?

- Two participants/caregivers described LTFU as a factor in college decisions. In one case, a patient's choice of college was accommodated with help from a parent and friends who assisted with subcutaneous injections for at-home treatment. In another case, a patient's initial choice of an out-of-state college requiring a new local care team did not work out, leading her to transfer to a college closer to her original treatment center.

15. Follow-up: Would you be able to get the same peace of mind if you were able to have your CAR T follow-up appointments at your local oncology sites rather than a primary center?

- Most participants who responded indicated they would still prefer main LTFU to remain with their original treating center and would prefer to have their specialist team assess the results from their LTFU visits, citing trust, continuity, and confidence in their specialists' expertise.
- Several participants noted they already receive certain routine services (e.g., lab work, scans) at local facilities that are part of their treatment center's broader network and described this arrangement as convenient without compromising their confidence in the results.
- One participant expressed skepticism that receiving care from local, non-specialist providers would provide the same peace of mind as care from her original pediatric/CAR T specialty center.

16. Follow-up: How was your enteritis side effect of CAR T diagnosed — by your local provider, or did you have to go back to your CAR T center?

- One participant described being diagnosed through the same care team that provided his CAR T treatment, since his local oncology care had already been transitioned to the transplant/CAR T team prior to treatment; his symptoms were eventually confirmed via colonoscopy and endoscopy after an initial period of diagnostic uncertainty.

FDA Offices & Divisions in Attendance

- Office of the Commissioner (OC) – 1 office
    - OC/OEA/PES – Office of External Affairs/Public Engagement Staff (organizer)
- Oncology Center of Excellence – 1 office
- Center for Biologics Evaluation and Research (CBER) – 15 offices
    - CBER/OCD – Office of the Center Director
    - CBER/OTP – Office of Clinical Evaluation
    - CBER/OTP/OCE/DCEGM/GMB2 – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation General Medicine/General Medicine Branch 2
    - CBER/OTP/OCE/DCEGM/GMB4 – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Clinical Evaluation General Medicine/General Medicine Branch 4

    - CBER/OTP/OCE/DCEH – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Hematology
    - CBER/OTP/OCE/DCEH/BHB – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Hematology/Benign Hematology Branch
    - CBER/OTP/OCE/DCEH/MHB – Office of Therapeutic Products/Office of Clinical Evaluation/Division of Hematology/Malignant Hematology Branch
    - CBER/OTP/OCE/OCTHT/DCT2/TEB1 – Office of Therapeutic Products/Office of Cellular Therapy and Human Tissue CMC/Division of Cell Therapy 2/Tissue Engineering Branch 1
    - CBER/OTP/OCE/OGT/DGT2 – Office of Therapeutic Products/Office of Gene Therapy Chemistry, Manufacturing and Controls (CMC)/Division of Gene Therapy 2
    - CBER/OTP/OGT/DGT2/GTB4 – Office of Therapeutic Products/Office of Gene Therapy Chemistry, Manufacturing and Controls (CMC)/Division of Gene Therapy 2/Gene Therapy Branch 4
    - CBER/OTP/OGT/DGT2/GTB5 – Office of Therapeutic Products/Office of Gene Therapy Chemistry, Manufacturing and Controls (CMC)/Division of Gene Therapy 2/Gene Therapy Branch 5
    - CBER/OTP/ORMRR – Office of Therapeutic Products/Office of Review Management and Regulatory Review
    - CBER/OTP/ORMRR/DRMRR1/RPMB1 – Office of Therapeutic Products/Office of Review Management and Regulatory Review/Division of Review Management and Regulatory Review 1/Regulatory Project Management (RPM) Branch 1
    - CBER/OTP/PSPS – Office of Therapeutic Products/Policy and Special Programs Staff
    - CBER/OVRR/DRMRR/RRB3 – Office of Vaccine Research and Review/Division of Review Management and Regulatory Review/Regulatory Review Branch 3
- Center for Drug Evaluation and Research (CDER) – 1 office
    - CDER/OND – Office of New Drugs
- Center for Devices and Radiological Health (CDRH) – 6 offices
    - CDRH/OPEQ/OHTI/DHTIA – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology IA
    - CDRH/OPEQ/OHTI/DHTIC – Office of Product Evaluation and Quality/Office of Health Technology I/Division of Health Technology IC
    - CDRH/OPEQ/OHTIII – Office of Product Evaluation and Quality/Office of Health Technology III
    - CDRH/OPEQ/OHTIII/DHTIIIA – Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology IIIA

    - CDRH/OPEQ/OHTIII/DHTIIIB – Office of Product Evaluation and Quality/Office of Health Technology III/Division of Health Technology IIIB
    - CDRH/OPEQ/OHTIV/DHTIVA – Office of Product Evaluation and Quality/Office of Health Technology IV/Division of Health Technology IVA

Non-FDA Attendees

- Reagan-Udall Foundation for the FDA

Financial Interest

Participants did not identify financial interests relevant to this meeting and are not receiving compensation for participation in this listening session.