American Society of Gene + Cell Therapy

Division of Dockets Management
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Re: Comments for Docket No. FDA-2025-N-0816 "Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments"

Dear Sir/Madam:

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to submit comments as the U.S. Food and Drug Administration (FDA) considers reauthorization of the Prescription Drug User Fee Act (PDUFA) for fiscal years 2028 through 2032. ASGCT is a nonprofit professional membership organization comprised of more than 6,400 scientists, physicians, patient advocates, and other professionals working on cell and gene therapies (CGT) in settings such as universities, hospitals, and biotechnology companies.

The PDUFA VII agreement represented a meaningful advancement for the field of cell and gene therapy. The enhanced review capacity at the Center for Biologics Evaluation and Research (CBER) has led to considerable improvements in the timeliness and consistency of regulatory reviews. These improvements have helped address the growing volume and complexity of CGT submissions. The addition of meeting types such as Type D and INitial Targeted Engagement for Regulatory Advice on CBER/CDER ProducTs (INTERACT), as well as the associated goals, has further supported more targeted engagement on novel scientific and regulatory questions. Our members have emphasized the value of these meetings in navigating emerging issues and facilitating more streamlined early development. Likewise, FDA's growing attention to modernized manufacturing methods and innovative technologies has been particularly important for the field. We appreciate the meetings and strategies put forward to date on these topics and encourage FDA to retain and build upon them.

As stakeholders begin planning for PDUFA VIII, ASGCT urges continued investment in CBER's capacity to support CGT reviews. Continued support for meeting infrastructure, regulatory science, and policy development within the Office of Therapeutic Products (OTP) will be essential to maintain momentum, including meeting goals. Members have also emphasized the importance of sustained attention to Chemistry, Manufacturing, and Controls (CMC) issues and advanced manufacturing technologies. Updating guidance on Good Manufacturing Practices (GMPs) and CMC expectations, particularly in the

context of platform technologies, would be a welcome area of focus in PDUFA VIII.

While not explicitly included in the PDUFA VII agreement, legislation passed in 2022 established pathways at FDA for designation of platform technologies and advanced manufacturing technologies.[1] These mechanisms have increased attention to these technologies and raised additional regulatory questions. We encourage PDUFA VIII to recognize the value of these designations and ensure FDA has the resources to review designation applications to reduce workload and increase efficiencies in the long term.

International regulatory alignment also remains a priority for ASGCT. We request FDA consider opportunities to deepen collaboration with international regulatory agencies such as the European Medicines Agency (EMA), Medicines and Healthcare products Regulatory Agency (MHRA), Health Canada, and the Pharmaceuticals and Medical Devices Agency (PMDA). Reducing duplicative requirements and exploring mutual recognition agreements for certain aspects of product review could accelerate global patient access and ease regulatory burden without compromising safety or quality. This would also be in alignment with the Agency's goals of reducing regulatory burdens.

ASGCT also supports the continuation and thoughtful expansion of several pilot programs launched under PDUFA VII. These include the Split Real-Time Application Review (STAR) pilot, the Rare Disease Endpoint Advancement (RDEA) pilot, and the CMC Development Readiness Pilot (CDRP). While ASGCT strongly supports the concept and structure of the RDEA pilot, members have noted that the submission process can be burdensome and that the pilot is not widely known. Given the critical importance of novel endpoint development in rare diseases, which represent a large proportion of gene therapy programs, we suggest FDA explore mechanisms to expand RDEA participation and improve its visibility. Likewise, the CDRP pilot has proven valuable in enabling earlier discussion of manufacturing strategies in accelerated development contexts. ASGCT recommends that this pilot be made permanent and used to inform a broader modernization of CMC expectations. The STAR pilot, modeled in part after the Oncology Center of Excellence's Real-Time Oncology Review (RTOR) program, offers opportunities to improve review efficiency and should be further evaluated for application beyond efficacy supplements.

ASGCT thanks FDA for its leadership in implementing the PDUFA VII commitment letter and for its continued efforts to modernize the regulatory framework for cell and gene therapies. If you have any questions, please do not hesitate to contact Margarita Valdez Martínez, Chief Advocacy Officer, at mvaldez@asgct.org.

Sincerely,

David Barrett, JD
Chief Executive Officer
American Society of Gene & Cell Therapy

[1] Food and Drug Omnibus Reform Act of 2022, Pub. L. No. 117–328, div. FF, §§ 2503–2504, 136 Stat. 4459, 5686–89 (2022).

20800 Swenson Drive, Suite 300 | Waukesha, WI 53186 | 414.278.1341 | asgct.org