July 7, 2025

U.S. Department of Health and Human Services
Office of the Secretary
200 Independence Avenue, SW
Washington, D.C. 20201

RE: Comments for Docket ID. AHRQ–2025–0001 "Request for Information (RFI): Ensuring Lawful Regulation and Unleashing Innovation to Make American Healthy Again."

Dear Sir/Madam:

The American Society of Gene & Cell Therapy (ASGCT) respectfully submits comments in response to the Department of Health and Human Services' (HHS) Request for Information (RFI): "Ensuring Lawful Regulation and Unleashing Innovation to Make America Healthy Again," which implements the President's deregulatory mandate under Executive Order 14192, *"*Unleashing Prosperity Through Deregulation.*"*

ASGCT is a nonprofit professional membership organization comprised of more than 6,400 scientists, clinicians, and patient advocates dedicated to the discovery and delivery of transformative cell and gene therapies (CGTs). ASGCT's strategic vision is to be a catalyst for bringing together diverse stakeholders to reshape the practice of medicine by incorporating the use of these transformative therapies. Many of ASGCT's members have spent their careers in this field performing the underlying research that has led to today's robust pipeline of CGTs. In Q1 2025, 4,418 advanced therapy candidates were in development, from pre-clinical stage through pre-registration, including 2,154 gene therapies and 966 non-genetically modified cell therapies.[1] Our members rely on clear, science-based FDA guidance to design development programs that are safe, efficient, and capable of bringing advanced therapies to patients without delay.

The Society supports the retirement of rules or practices that no longer serve a meaningful public-health purpose. CGT science is advancing at an unprecedented pace, evidenced by the 79 new gene therapy clinical trials initiated in Q1 2025,[1] the highest quarterly total in the past year. In the same quarter, six U.S.-headquartered CGT biotech start-ups secured early-stage financing to move their first programs toward the clinic. Small biotechs like these, often operating with limited resources, depend on timely and

[1] Gene, Cell, + RNA Therapy Landscape Report, American Society of Gene & Cell Therapy and Citeline, [Q1 2025]

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

up to date FDA guidance that sets clear expectations for pathways such as Accelerated Approval, delineates acceptable chemistry, manufacturing, and controls (CMC) strategies, and explains how platform data can be leveraged across programs.

While the "10-for-1" deregulation mandate may be appropriate for other fields, ASGCT supports the publication of guidance documents that FDA's Center for Biologics Evaluation and Research (CBER) has slated for release in 2025. CBER's publicly posted 2025 Guidance Agenda identifies several documents that are indispensable to the day-to-day work of CGT developers, including final guidance on *Potency Assurance for Cellular and Gene Therapy Products; Safety Testing of Human Allogeneic Cells Expanded for Use in Cell-Based Medical Products; Considerations for the Use of Human- and Animal- Derived Materials and Components in the Manufacture of Cell and Gene Therapy and Tissue-Engineered Medical Products*, and the long-awaited draft guidance on *Accelerated Approval of Human Gene Therapy Products for Rare Diseases* and on *Use of Platform Technologies in Human Gene Therapy Products Incorporating Human Genome Editing*. Each of these addresses recurring scientific questions that, now left unanswered, require sponsors to seek case-by-case advice through formal meeting requests, lengthening the FDA review period, and ultimately delaying the delivery of transformative therapies to patients.

In order for developers to address unmet patient needs, as evidenced by the robust pipeline for CGTs in rare diseases and common diseases, the Society encourages FDA to develop new standardized approaches that will reduce the scientific uncertainty and accelerate the regulatory pathway of each new product.[2] Entering 2026 with clear guidance on validating potency assays, bridging data across genome-editing platforms, and designing confirmatory studies for accelerated approvals will keep promising therapies on track. Even modest reductions in uncertainty will let sponsors allocate resources more strategically, modernize manufacturing with confidence, and maintain steady momentum across their development programs.

While new guidance may be needed in some instances, there are also opportunities to expand existing FDA resources. FDA's *Frequently Asked Questions – Developing Potential Cellular and Gene Therapy Products* draft guidance notes it may be periodically updated with additional FAQs. In the Society's comments to the draft guidance, we encouraged the Agency to expand this resource on a predictable schedule, so new questions can be addressed without reopening the entire guidance process. Converting high-traffic CGT guidances to living resources with a Federal Register notice summarizing revisions would satisfy sponsors' need for timely clarity while aligning with the Administration's deregulatory goal of eliminating outdated language and expectations.

Finally, the Society sees a valuable opportunity to reduce many of the burdens noted in the RFI by more closely aligning established policy tools with the existing regulatory framework. The Advanced Manufacturing Technologies (AMT) Designation Program and the Platform Technology Designation Program were created by Congress, under the Food and Drug

[2] Ibid.

Omnibus Reform Act (FDORA) in 2022,[3] to accelerate innovation, yet both are constrained by the final rule Biologics License Applications and Master Files (89 FR 9743) ('BLA DMF rule'), which prevents sponsors from referencing previously reviewed manufacturing information. ASGCTs previous comments on both programs explain why harmonizing the rule with these pathways would reduce duplicative submissions and FDA workload while preserving robust oversight. We encourage HHS to prioritize such targeted fixes over indiscriminate rescission.

Timely, science-based guidance benefits patients and innovators alike by providing clear regulatory expectations. We therefore urge HHS to ensure that any deregulatory plan preserves, and ideally strengthens, FDA's capacity to issue such guidance for CGTs. ASGCT's scientists and professionals stand ready to collaborate on achieving a balanced approach that streamlines bureaucracy while sustaining the clear regulatory roadmap essential to patient safety and therapeutic progress.

Thank you for the consideration of these comments. 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

[3] U.S. Congress. (2023). *Consolidated Appropriations Act (H.R.2617).* https://www.congress.gov/bill/117th-congress/house-bill/2617