Fiscal Year 2027 Food and Drug Administration Appropriations
House Subcommittee on Agriculture, Rural Development, FDA, and Related Agencies
David Davenport, Director, US Policy, Alliance for Regenerative Medicine
May 1, 2026

Chairman Harris, Ranking Member Bishop, and distinguished members of the Subcommittee, the Alliance of Regenerative Medicine (ARM) appreciates the opportunity to submit testimony on the U.S. Food and Drug Administration (FDA)'s fiscal year (FY) 2027 appropriations. ARM is concerned that late-stage shifts in evidentiary expectations for cell and gene therapies (CGTs) at the FDA over the past year have created growing uncertainty for patients, biotechnology companies, clinicians, and investors, and risks delaying or eliminating treatments for patients with progressive diseases who often have no other options. Sufficient staffing, resources, training, and leadership at FDA are vital to ensuring predictable regulatory oversight, Americans' timely access to transformative treatments, and the U.S.'s leadership in CGT innovation. **ARM supports increasing the overall funding level at the FDA. For the Center for Biologics Evaluation and Research (CBER) that regulates CGTs, we recommend Congress at least maintain the FY 2026 funding level of $601M and consider how reductions in budget authority would impact needed hiring, training, and resources as the FDA manages a growing and complex workload for CGTs.**

ARM is the leading international advocacy organization championing the benefits of cell and gene therapies for patients, healthcare systems, and society. As the global voice of the sector – representing more than 400 member organizations including biotechnology companies, academic and medical research institutions, and patient organizations – we recognize the indispensable role the FDA plays in bringing groundbreaking therapies to patients expeditiously while maintaining the highest standards of safety and efficacy.

CGTs transform how we treat some of humanity's most devastating diseases and provide

hope to patients who face death or serious disability. Unlike traditional pharmaceuticals, CGTs target the root cause of disease at a cellular or genetic level and can provide years and sometimes even a lifetime of medical benefit, often with a single administration. The U.S. has introduced more CGT products into the market than any other country and is a world leader in promoting access to CGTs. To date, the FDA has approved 50 CGTs.[i] Research has projected approvals of between 75 and 96 new CGT product-indications by 2033,[ii] with estimates of the number of patients receiving CGT treatments increasing approximately tenfold in a similar period.[iii] FDA-approved CGTs have already transformed the lives of thousands of patients, many of them children, with aggressive blood cancers and serious rare diseases such as sickle cell disease, hemophilia, and spinal muscular atrophy. These therapies also reduce the need for hospitalization and chronic interventions, which for many diseases with approved CGT treatments can reach several million dollars over a lifetime.

A course-correction at the FDA is urgently needed for late-stage CGT programs

The Trump Administration, including HHS and FDA leadership, have publicly stated unprecedented support for accelerating CGT development to ensure America remains a global biotech leader in this rapidly growing field. In 2025, the FDA convened a CGT roundtable and listening sessions with biotech CEOs. Over the past year, the FDA has also proactively outlined multiple new policies to advance the CGT sector, such as the Rare Disease Evidence Principles program; a new Plausible Mechanism Framework for individualized therapies; and flexibilities for chemistry, manufacturing, and controls (CMC) requirements.

Despite these actions and the recent approval of a gene therapy for genetic hearing loss, the FDA has overwhelmingly refused to file or declined applications for multiple promising CGT medicines – including treatments for rare diseases – by issuing complete response letters

(CRLs). Particularly concerning is the reversal of previous agency guidance and inconsistent application of regulatory flexibilities for these rare disease products, despite the fact that nearly all of them received Regenerative Medicine Advanced Therapy (RMAT) designation. These decisions have included the rejection of endpoints or development strategies that had previously been discussed with FDA, and FDA pushing for randomized trial requirements in rare disease settings where they may be infeasible or unethical. There has also been reduced use of Advisory Committees to help resolve scientific disagreements and consider input from patients.

Collectively, these decisions have raised concerns over regulatory clarity and predictability and undermined the stated goals of HHS and FDA leadership to accelerate CGT development. Across the CGT sector, stakeholders are increasingly concerned that FDA review practices are moving toward a more rigid interpretation of clinical evidence that does not reflect the realities of rare disease development or leverage regulatory flexibilities granted by Congress, such as RMAT designation and accelerated approval. By making RMAT-designated products eligible for accelerated approval, for example, the RMAT program encourages the use of flexible clinical trial designs, novel endpoints, and real-world evidence. These actions also come at a time when China is racing to eclipse the US as the global leader in CGTs: During H2 2025, clinical trials for CGTs grew 20% in China following efforts to attract early-phase proof of concept trials to generate in-human data quickly, compared to only 8% in the United States.

The Committee should work with the FDA to ensure that practical and actionable steps are taken to address growing concerns over regulatory inconsistencies and late-stage shifts in guidance for CGTs and to ensure that the regulatory flexibilities Congress has authorized are meaningfully and consistently used. Specifically, FDA should:

(1) *Restore predictability in development pathways*: Multiple companies which had

previously aligned with FDA on development plans now face regulatory decisions that effectively reset development timelines by at least several years. The FDA should honor the direction originally given to companies and patients about evidence acceptable for approval.

(2) *Apply fit-for-purpose evidentiary standards for rare disease therapie*s: Rare disease development often requires flexible, fit-for-purpose approaches to clinical evidence when randomized trials are infeasible or unethical. To prevent unnecessary delays, the FDA should leverage post-market commitments to further establish efficacy after approving the therapies.

(3) *Improve transparency in communication by convening FDA Advisory Committees to resolve scientific disagreements*: When disputes arise regarding endpoints or evidentiary standards, these issues should be addressed through transparent scientific dialogue with sponsors, experts, and patient communities rather than resolved solely through regulatory actions. Upon request from the sponsor, FDA should assemble outside experts through an FDA Advisory Committee to review these therapies and listen to testimony from patients about benefit-risk considerations. If a sponsor requests an Advisory Committee meeting, one should be granted.

(4) *Select a new CBER Director who understands drug development and regulation*: A new head of CBER should be selected who truly understands drug development, including for rare diseases; brings a philosophy and culture to CBER that supports resolving issues early to facilitate drug development; and has strong management experience.

Without a course correction, patients with rare diseases and other unmet medical needs will face longer delays in accessing life-saving therapies, investment in innovative treatments will decline or shift outside the United States, and U.S. leadership in advanced biomedical innovation will be undermined.

Sufficient staff and training resources are essential to predictable regulatory oversight

The FDA's Center for Biologics Evaluation and Research (CBER), and specifically the Office of Therapeutic Products (OTP), have played a critical role in providing scientific and regulatory leadership to advance CGTs. Training for human drug/biologics reviewers takes approximately 3-5 years, and with nearly 900 ongoing clinical trials for CGTs in the U.S., CBER must be prepared to manage a growing and complex workload for CGTs that represent tremendous hope patients and their families – not only for those suffering with rare diseases, but also common ones like heart disease, Parkinson's disease, and diabetes.

Since January 2025, however, CBER has seen a 22.5% reduction in staff;[iv] turnover in senior level staff positions – including the role of CBER Director; decreases in staff training; and decreased external engagement including through Advisory Committees. High staff turnover requires sufficient staff and funding for training and external continuous learning as well as intentional team building. Budget authority also provides the FDA with the broadest and most flexible means of meeting all of its responsibilities and would allow the agency to focus on providing near- and long-term regulatory clarity for CGTs.

As the Committee considers the FDA's FY 2027 appropriation, we urge Congress to prioritize hiring through budget authority, establish a workforce hiring and training plan, and ensure sufficient funding and staff resources are provided to fully implement regulatory flexibilities authorized by Congress. A strong leadership and workforce at CBER and OTP are essential to maintaining FDA's ability to evaluate complex and diverse CGTs consistently, efficiently, and effectively.

---

[i] https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products.
[ii] https://newdigs.tuftsmedicalcenter.org/payingforcures/defining-disruption/cell-and-gene-therapy-products-and-%20pipeline/cgt-pipeline-deep-dive/#gsc.tab=0.
[iii] https://www.sciencedirect.com/science/article/pii/S1359644625002648.
[iv] https://www.fda.gov/industry/fda-user-fee-programs/center-drug-evaluation-and-research-center-biologics-evaluation-and-research-net-hiring-data-fy-2023