Equipping the FDA to Advance Gene Therapies

IGT
Institute for Gene Therapies

Gene therapies are changing the future of human health as we know it. After more than 20 years of careful study, gene therapies are now making their way through the FDA approval process and to patients who need them.

By targeting the cause of disease directly, gene therapies have the potential to fundamentally reshape the trajectory of disease. Gene therapies can offer long-lasting – sometimes lifelong – benefits for patients and the healthcare system.

Our constantly evolving knowledge of gene therapy development and manufacturing necessitates flexible, innovative approaches to bring safe and effective therapies to patients with urgency.

Adequate resources and expertise are essential for ensuring the FDA's ability to perform its mission.

The Center for Biologics Evaluation and Research (CBER), responsible for oversight of gene therapies, has acknowledged the challenges of keeping pace with the new science and increased development of gene therapy products. Gene therapy is a rapidly evolving field with limited precedent, and many issues must be addressed on a case-by-case basis. As such, the FDA must be prepared in a multitude of ways to engage in timely, effective dialogue with sponsors. **IGT calls on Congress to encourage CBER to further leverage existing expertise within and across Centers and divisions (e.g. disease-specific knowledge) to ensure appropriate funding and staffing allocations for CBER for years to come.** Specifically, we ask Congress to:

APPROPRIATE new funds to FDA for FY 2025

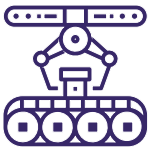
SUPPORT FDA in implementing innovative and flexible tools and trial designs to advance gene therapies

ENCOURAGE FDA to leverage existing expertise internally across Centers and externally

Preserving and Enhancing Expedited Programs

The FDA's expedited programs, including the Regenerative Medicine Advanced Therapy (RMAT) designation and accelerated approval pathway, deliver life-enhancing and life-saving new treatments, such as gene therapies, to patients with serious, life-threatening diseases quickly. The FDA has indicated that the accelerated approval pathway can be an appropriate avenue for gene therapies that offer the potential to alter or cure the underlying genetic defect that causes a serious disease not addressed by available therapy. Congress should encourage FDA to utilize, and enhance, expedited programs, such as RMAT and accelerated approval, when appropriate to ensure gene therapies reach patients in a timely way.

IGT is concerned that payers are increasingly denying or restricting access to medicines approved through expedited approval programs undermining FDA's effectiveness and efficiency in delivering innovative therapies to seriously ill patients with significant unmet needs. **IGT asks that Congress reinforce FDA's statutory role as the expert entrusted by Congress to determine the safety and efficacy of medicines for patients in the U.S.**



Improving Manufacturing Flexibility

FDA has made great strides in developing flexible approaches to advance clinical development of treatments for serious or life-threatening conditions. For gene therapy, compressed clinical development can lead to a bottleneck in Chemistry Manufacturing and Controls (CMC) aspects of development, potentially delaying the availability of urgently needed treatments. Gene therapy manufacturing is extremely complex and highly dynamic, evolving as experience grows. This is particularly true for treatments addressing rare diseases with limited patient populations. **IGT calls on Congress to enable FDA to apply an iterative, risk-based CMC review framework that considers disease severity, rarity and unmet need, stage of development, and prior knowledge from related manufacturing processes.** This will help expedite development and ensure timely access to life-saving therapies while maintaining patient safety and FDA's rigorous approval standards.

Facilitating Individualized Gene Therapy Pathways

Many serious and life-threatening diseases have no current treatment options or standard of care and are very rare, affecting as few as one patient globally. A new, streamlined regulatory process is needed to create a sustainable path to approval for individualized gene therapies which may treat a very small number of patients ranging from ~1 to 100/year and otherwise might not be developed. **IGT urges Congress to support FDA's efforts to recognize the unique advantage of gene therapy platforms – namely, the ability to leverage data and information from one gene therapy to another when common components (e.g., vector, promoter) are used across therapies and disease areas.**

Absent a new pathway, Congress should implore FDA to allow innovators to utilize modern clinical trial approaches, such as adaptive, integrated, and combination designs. Traditional phase 3 randomized control trial approaches are often untenable for rare diseases where patient populations are small and treatment centers are few. Failure from FDA to allow manufacturers to rely on modern designs will yield nothing for patients who will be forced to continue waiting to see treatments.