July 22, 2026

Department of Health and Human Services
Food and Drug Administration
[Docket No. FDA-2026-N-4699]

**RE: Comments on Expedited Investigational New Drug Program; Request for Information**

We share the Food and Drug Administration (FDA)'s commitment to accelerating the development of therapies and cures for the American people and appreciate the opportunity to comment on its proposal to establish the Expedited Investigational New Drug (IND) pilot program.[1] We applaud and support the FDA's proactive approach to advancing our global regulatory leadership and facilitating the continuation of early-stage research in the U.S. by streamlining requirements and innovating processes. We believe the proposed pilot program is a valuable step, but its greatest impact will come if it is implemented alongside broader modernization of FDA's early clinical development framework.

I. The Importance of Maintaining U.S. Leadership in Clinical Research

For decades, the United States has been the world's leading hub for biomedical innovation, supported by a uniquely productive ecosystem of scientific research, biotechnology, and pharmaceutical companies, mature regulatory frameworks, venture investment, and clinical development infrastructure. In recent years, its clinical research infrastructure has supported the largest share of global clinical trial activity among individual countries.[2] This leadership has produced remarkable advancements in novel medicine products that hold significant promise for American patients, from targeted therapies and regenerative medicines to increasingly sophisticated diagnostics.

Maintaining this leadership is critical for the following reasons:

1. It accelerates the development of new treatments for patients.
2. It helps ensure that research addresses the diseases, populations, and public health priorities most relevant to Americans.
3. It expands opportunities for U.S. patients to participate in clinical trials, providing earlier access to promising investigational therapies.
4. It attracts and retains world-class scientists, clinicians, and entrepreneurs, which reinforces the United States' capacity for future innovation.
5. It strengthens the U.S. economy by creating high-value jobs, boosting economic activity, and driving investment. For example, in 2022, biopharmaceutical manufacturers contributed more than $1.65 trillion to the U.S. economy directly through supplier activity.[3]

While the United States remains the global leader in industry-sponsored clinical research, a growing share is moving to countries that are perceived as offering more efficient regulatory pathways for early-phase

[1] The opinions expressed in this document are those of its authors as individuals and do not represent the opinions or positions of the University of Southern California or the USC Schaeffer Institute.
[2] IQVIA Institute for Human Data Science. Rethinking Clinical Trial Country Prioritization. Published online July 2024. https://www.iqvia.com/insights/the-iqvia-institute/reports-and-publications/reports/rethinking-clinical-trial-country-prioritization
[3] PhRMA. New: Research: Industry sponsored clinical trials contribute billions to state economics. Published online March 6, 2025. https://phrma.org/blog/new-research-industry-sponsored-clinical-trials-contribute-billions-to-state-economies

studies, such as China and Australia.[4] According to industry pipeline data, 46% of all new drug molecules entering first-in-human clinical trials in the first half of 2025 originated with Chinese companies.[5] This reflects a remarkable shift in the global innovation landscape as, just five years ago, U.S. pharmaceutical companies did not license any new drugs from China, but by 2024, one third of the new compounds they licensed came from Chinese biotechnology firms.[6]

## II. The Expedited IND Pilot Should Be Part of a Broader Modernization Strategy

Strategic reforms at the FDA can strengthen the competitiveness of the United States as a destination for biomedical research and investment, while also improving patient access to safe and effective medical products. The Expedited IND pilot program can be an important part of these efforts, but it should be considered just one component of a broader set of reforms. Moreover, as a pilot program with multiple novel features, its impacts are likely to take longer to materialize than those of other reforms that the agency might consider. For this reason, however the FDA chooses to proceed with this pilot, we encourage the agency to prioritize complementary efforts that will have broader and more immediate impacts on the regulatory climate for early-stage clinical research in the United States.

### Update Legacy Guidance Documents

First, we strongly encourage the FDA to prioritize updating guidance documents that have become outdated over time. For example, the FDA's guidance document on INDs for Phase 1 studies was written in 1995,[7] and its guidance on clinical holds was written in 2000.[8] Although they were originally written to provide a flexible approach to data expectations, the age of these documents creates unnecessary burdens for researchers trying to understand the agency's current thinking and approach. When the relevant guidance has not been updated for decades, sponsors often rely on informal interpretations or historical interactions with FDA staff that may no longer reflect current agency thinking. Updating these documents would provide an opportunity to ensure that stakeholders are aligned in their understanding that agency guidance reflects current policy, and it would provide a procedural vehicle for advancing helpful policy updates that have been discussed as part of Operation TrialBlazer, such as streamlining data requirements in areas such as pharmacology, toxicology, and chemistry, manufacturing, and controls (CMC).[9]

### Support Seamless Clinical Development

We also encourage the FDA to prioritize building the technical and regulatory infrastructure needed to support broader use of seamless clinical trial designs, in which multiple phases of development are conducted under a single, overarching protocol with pre-specified criteria for progressing from one stage to the next. By reducing the need to pause development and initiate new protocols between trial phases, seamless designs can shorten development timelines, lower costs, and improve regulatory efficiency. They can also advance the objectives of Operation TrialBlazer by making it easier to conduct clinical

[4] IQVIA Institute for Human Data Science. Rethinking Clinical Trial Country Prioritization. Published online July 2024. https://www.iqvia.com/insights/the-iqvia-institute/reports-and-publications/reports/rethinking-clinical-trial-country-prioritization
[5] Goldman Sachs. China is Increasing Its Share of Global Drug Development. Published online December 17, 2025. https://www.goldmansachs.com/insights/articles/china-is-increasing-its-share-of-global-drug-development
[6] Gottlieb, S. How to stop the shift of drug discovery from the U.S. to China. STAT. May 6, 2025. https://www.statnews.com/2025/05/06/how-to-stop-the-shift-of-drug-discovery-from-the-u-s-to-china/
[7] CDER & CBER. Guidance for Industry. November 1995. https://www.fda.gov/media/71203/download
[8] CDER & CBER. Guidance for Industry. October 2000. https://www.fda.gov/media/72548/download
[9] https://www.hhs.gov/sites/default/files/operation-trialblazer.pdf

development as an integrated U.S. program. When Phase 1 through Phase 3 development is planned as a continuous program under a single protocol, sponsors are less likely to bifurcate their clinical development by conducting first-in-human studies abroad. We are therefore encouraged by the FDA's recent announcement that the agency is building its capacity to support real-time reporting of trial results,[10] which represents an important step in building the infrastructure needed to support seamless development programs.

**Prioritize Broader Regulatory Modernization**

We encourage the agency to continue prioritizing regulatory reforms that will help the United States maintain its leadership in later-stage clinical research. For example, the FDA should:

- build on its progress facilitating novel trial designs—including adaptive trials and master protocols—that let sponsors continue or modify development based on accumulating data rather than restarting the process at each phase;
- expand its programs to provide meetings and individual guidance to developers who are using innovative approaches to evidence generation;
- extend FDA's approach to patient perspective data for devices to all medical products, so that developers are encouraged to include such data throughout the product lifecycle, particularly in clinical trial design, to ensure that studies are evaluating what matters most to patients,[11] and
- encourage the appropriate use of external control arms (ECAs) by updating guidance to help product developers better understand the benefits of using ECAs and identify appropriate use cases. Updated guidance could encourage more use of ECAs when randomization is infeasible or unethical, reduce unnecessary trial burden, and expand patient access to investigational therapies.

**Conclusion**

We appreciate the FDA's leadership in exploring new approaches to accelerate clinical development through the Expedited IND pilot. We encourage the agency to view the pilot as one element of a broader modernization effort that includes updated guidance, greater support for innovative trial designs, and continued efforts to improve regulatory efficiency while maintaining FDA's longstanding standards for safety and scientific rigor. Together, these reforms can help ensure that the United States remains the world's leading environment for developing innovative medical products and delivering new therapies to patients.

We appreciate the opportunity to comment on this proposed pilot and would welcome the opportunity to discuss these recommendations further with HHS/FDA leadership.

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[10] https://www.fda.gov/news-events/press-announcements/fda-announces-major-steps-implement-real-time-clinical-trials
[11] FDA/CDRH. Incorporating Voluntary Patient Preference Information over the Total Product Life Cycle. Published online March 27, 2026. Accessed March 27, 2026. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle-0