WRITTEN TESTIMONY OF
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"Maintaining America's Leadership in Biomedical Innovation: FDA's Role in Advancing U.S. Drug Development"

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Introduction

Chairman Griffith, Ranking Member DeGette, and Members of the Subcommittee, thank you for the opportunity to submit this testimony. I am Susan Winckler, and I serve as Chief Executive Officer of the Reagan-Udall Foundation for the Food and Drug Administration (the Foundation). I am a pharmacist and an attorney by training, and I have had the privilege of viewing drug development from several vantage points across a thirty-year career — including service as Chief of Staff at the FDA from 2007 to 2009, where I supported both Republican and Democratic Commissioners.

Maintaining America's leadership in biomedical innovation is one of the defining public-health challenges of this decade, and the FDA plays a significant role in addressing that challenge. This testimony draws on select projects from the past year where the Foundation convened the people who develop drugs and run trials. I hope to leave you with confidence in the direction our system

is heading and, more usefully, a clearer picture of the work that remains not only for FDA, but for the broader clinical trials enterprise.

The Foundation is an independent, 501(c)(3) nonprofit organization created by Congress in the FDA Amendments Act of 2007, with the purpose of advancing regulatory science and supporting the FDA's mission.[1] Congress named our organization for President Ronald Reagan and Congressman Morris "Mo" Udall, each of whom lived with an incurable neurodegenerative disease — a reminder that our work is about addressing challenges, accelerating innovation, and improving patients' lives. Consistent with the parameters of our authorizing statute, the Foundation does not engage in regulatory activity nor regulatory decision-making.

I provide this background of our organization's role because it shapes what we can offer this Subcommittee. The Foundation is a convener, not an advocate. We provide a trusted, transparent forum where relevant FDA ecosystem participants—in this case clinical trial sites, clinical research organizations, regulated industry, patient organizations, and regulators—can step above individual product decisions and discussions and engage, with candor, about a topic—in this case the shared challenges, data gaps, and inefficiencies that challenge America's leadership in biomedical innovation. Described another way, we elevate the conversation to a level of common exploration and problem-solving. Efficiencies are gained when parties collaborate rather than each evaluating

[1] The Reagan-Udall Foundation for the FDA is supported by federal funding and private donations. Our operations are partially supported by statutorily-required funding from the U.S. Food and Drug Administration (21 USC Chapter 9, Subchapter VII, Sec. 379dd(n)). All programmatic decisions are our own. We maintain strict oversight through our Board of Directors, which reviews all financial donations over $250 and rejects gifts that pose a conflict of interest or other concern. The Foundation embraces transparency and prioritizes safeguards, including annual reporting to the public, Congress, the FDA, and the IRS. These measures protect against undue influence and ensure independence, accountability, and scientific integrity.

the same problem alone. My testimony is offered in that spirit: a faithful report of what stakeholders have told us, and where they have reached agreement.

Thus, I appear today not to advocate for any specific legislation, but to share what our convenings have surfaced and provide observations useful to the Subcommittee's deliberations.

How These Reports Were Generated

As a component of our activity across FDA's broad jurisdiction, the Foundation assembled stakeholders to explore three distinct areas of drug development: one focused on enhancing early-stage drug development in the U.S., another discussing improvements in large multi-regional clinical trials with a focus in oncology, and a third exploring the gap between patient-relevant and regulatorily-sufficient endpoints in developing products to address rare disease. Each convening involved different participants from the broad FDA ecosystem, and the learnings from each discussion are captured in individual reports. I personally moderated each roundtable discussion and can attest that the participants came to each table with sharply different interests, no shared script, and a willingness to engage with each other constructively. Together, the participants produced dozens of specific, stakeholder-vetted recommendations that serve as an array of potential activities for all in the ecosystem to explore, revise, and pursue.[2] This testimony will capture some areas of alignment between potential activities and recently announced initiatives, and will highlight areas where more might be done.

[2] See page 12 for links to reports referenced in this testimony.

A Foundation for Confidence

When the Department of Health and Human Services released Operation TrialBlazer, a roadmap for maintaining U.S. leadership in early clinical research and development, our team compared each of those three bodies of stakeholder work against the roadmap. Across three separate convenings, we found our report recommendations converge repeatedly and substantively with the direction HHS has charted. That convergence is not coordination; these were independent efforts. When the people who run trials, treat patients, and generate the evidence arrive on their own at the same conclusions as the agency that regulates them, it is a strong signal that the reforms are sound and ready to implement.

To name the strongest alignments briefly: in early-stage development, both our stakeholders and HHS embrace risk-based nonclinical testing and streamlined Phase 1 manufacturing expectations; in improving large, later-stage studies, both prioritize network trial models, single-IRB reliance, and patient-centric trial matching; and finally, our reports and Operation Trialblazer embrace modern adaptive trial designs and fuller use of real-world data. The Subcommittee should take confidence from that agreement. There are opportunities, however, that are not yet pursued and where this Subcommittee holds the tools to drive meaningful change.

A Word on Competitiveness

This hearing rightly frames biomedical innovation as a matter of American leadership. Allow me offer one piece of personal context. In 2007, as FDA Chief of Staff, I led the agency's medical-product negotiations with China's then-State Food and Drug Administration, which produced a bilateral product-safety agreement between the two countries. In that process, I was immersed in the reality that U.S consumers and patients are best served when regulatory authorities collaborate.

At the time, the U.S. was challenging China to enhance their regulatory agility and maturity, to help that country better oversee medical product safety and better harness innovation. Since then, China has done just that: they have developed a stronger and more efficient medical product regulatory system. And thus, China has joined other countries in competing for biomedical innovation, and the U.S. has the opportunity to further improve its performance to nurture that innovation.

I raise this for transparency, not to dwell on, or target, any other country. The Foundation takes no position on trade or geopolitics, and I observe that a durable response to global competition is not to define ourselves against anyone else, but rather to strengthen what we do here, to improve our own operations. Every one of our convenings was, at its core, about that strengthening: how the United States can make its own system faster, clearer, and more capable, so that innovation is developed here because here is the best place to develop it. That is a frame for the Subcommittee to consider: the goal is a stronger American system, and the surest way to lead is to be the best place in the world to bring a therapy from discovery to patients.

Five Themes Where Consensus Runs Deepest

Reading the three convenings together with Operation Trialblazer, five themes recur consistently:

- **Clarity in communication.** Sponsors lose months guessing at FDA's expectations and defaulting to over-conservative data generation, often providing more data than is needed, which takes additional months to gather and analyze. This is especially damaging for smaller, emerging biopharma companies who have little capital to spare and can lead to some promising drug candidates being scrapped before clinical trials can start. Clear, phase-appropriate requirements were the single most cited reform across all three

convenings, and HHS places them first as well. And the FDA could go further: use of the phrase 'clinical hold' to describe the FDA decision to hold an investigational new drug application at the preclinical stage is, not surprisingly, misunderstood by many to mean that actual clinical work has been stopped. When this situation occurs prior to any patient receiving a dose and the action is informational, the descriptor "notice of additional information required" is simply clearer. Further, differences in evidentiary expectations among review divisions at the FDA are often raised as a challenge to product innovation. Sharing the underlying rationale for such differences, particularly the scientific principles driving disparate treatment, can be educational for many product developers and fellow regulators. Extending that such a culture of shared learning to the Agency's application of flexible evidentiary strategies would be an additional innovation acclerant.

- **Reducing duplication.** Single-IRB reliance, reusable regulatory playbooks, computable protocols, and reuse of prior manufacturing knowledge each address redundant effort that adds cost and delay without adding commensurate protection for patients or participants.

  In addition, there is opportunity for regulated industry to consider its role in "over-answering" or borrowing from precedent when unnecessary. As FDA provides greater clarity on what is actually required, industry must review, and apply, that clarity. (See Early-Stage Drug Development Recommendation #12.) Learning forums to share information about pre-IND meetings, IND submission packages, and IND reviews can yield discussion of what has worked, what has not, and where expectations may have shifted.

- **Modern, efficient trial designs.** Master protocols, adaptive and platform trials, and network models appear in every analysis as options to learn more from fewer patients and

less duplicated infrastructure. Doing more with less is essential in oncology and indispensable in rare disease where the patient population may be measured in dozens. Unlocking the potential of network (or hub-and-spoke) models can increase efficiency (and innovation) and allow broader participation of patients in clinical trials. (See Oncology Clinical Trials Recommendation #1)

- **Better use of existing data.** Real-world data, natural history, and patient registries are treated across all three convenings as regulatory assets, with a shared call for clearer, more predictable criteria on when and how such data sources and analysis can support a regulatory decision. Going a step further and pursuing a cross-stakeholder, AI-enabled rare disease knowledge management system could help maximize the impact of this inherently valuable rare data. (See Expanding Regulatory Agility and Evidentiary Integrity in Developing Treatments for Rare Diseases Recommendations #20)
- **An accessible FDA and early engagement.** New consultation channels, contact centers, plain-language resources, and public engagement reflect a shared belief that resolving scientific and regulatory questions early prevents far more costly rework later. A short, well-timed conversation may save a sponsor many months of investment. Broader conversations, where regulators and researchers share their learnings, accelerate the exploration of emerging ideas and, inherently, innovation.

The Work That Remains: Observations Offered for the Subcommittee's Consideration

Candor about alignment requires equal candor about the gaps, because those gaps are where the stakeholder community has completed informative preparatory work. I offer these not as criticism

of Operation Trialblazer, but as a map of where additional effort could accelerate solutions that industry and sites are ready to explore. Several of the not-yet-addressed opportunities identified by our stakeholders share a common feature: they cannot be solved by the FDA alone, and in some cases not by HHS alone. They require resources, statutory clarity, or infrastructure that only Congress can provide. Consistent with the Foundation's role, I offer the following as observations of strong stakeholder consensus— areas the Subcommittee may find worth its attention.

Improving Transparency and Efficiency of the US Clinical Trial Infrastructure

Much of the reform conversation, including Operation Trialblazer, focuses on the regulation of early stages of medical product development: what data an IND requires, how protocols are reviewed, how trials are activated. Those rules matter. But rules and regulations operate on an assumption of physical and human capacity to discharge against those rules. And that capacity is unclear. We do not have a clear, current nationwide picture of where capacity for first-in-human, Phase 1 trials actually resides; we do not have a resource to illustrate which academic medical centers, health systems, and dedicated units can safely and quickly initiate early-phase trials, what specialized capabilities they hold, and how much of that capacity is at risk from staffing shortages and financial strain. If we cannot see our own capacity, we cannot strengthen it, and no amount of regulatory streamlining will compensate for a first-in-human infrastructure that is thinning or non-existent. This challenge extends beyond Phase I trials: trial capacity is unevenly distributed across the country with many patients living far from any trial site. Furthermore, the number of trial sites is declining under financial pressure.[3]

[3] Friedson, Kim, "The Supply of Clinical Trial Sites: Openings, Closings, and Longevity", Milken Institute, September 2025.

A key recommendation from our reports includes building a dedicated network of Phase 1 capable clinical trial sites, including designating first-in-human/Phase I centers of excellence that apply key reforms such as a single IRB and other streamlined site activation processes. (See Early-Stage Drug Development Recommendation #14) Federal funds would clearly enhance such a network while building capacity, disseminating best practices, training the next generation of early-phase investigators, and extending reach into underserved regions. This, in turn, could be part of a national Phase I coordinating strategy.

I want to address one genuine difference in emphasis, because I think it is clarifying rather than concerning. Operation TrialBlazer is structured primarily from a posture of improving competitiveness, with a goal of reversing the trend of investors and sponsors looking overseas to hold their early-phase clinical trials. The Foundation's convenings were animated primarily by the patient: by what patients feel, function, and survive, and by how to reach them faster and more equitably. I do not see these as competing motivations. They are two roads to the same destination. A regulatory system that is faster, clearer, and less duplicative serves to keep innovation onshore and gets therapies to American patients sooner.

Another opportunity to explore is re-thinking the clinical trial structure in discrete disease areas. For example, consider pivoting from screening an individual patient against potential trial participation in a consecutive, trial-centric manner to a truly patient-centric approach. A common, pre-competitive platform that enables patients to be screened for a broad array of trials and referred to the trial(s) that best fit their needs helps patients *and* innovation. (See Oncology Clinical Trials Recommendation #2) The Subcommittee does not have to choose between the competitiveness case and the patient case; the same reforms serve both.

Each report supports the shift to risk-based, phase-appropriate nonclinical and manufacturing requirements, and consistently emphasizes that the value of that shift depends on the FDA finalizing the associated guidance documents so that clarity becomes durable rather than provisional. Stakeholders view the underlying data infrastructure, e.g., electronic health record integration with trial databases, computable protocols, and secure, interoperable real-world data, as the foundation that makes patient-centric enrollment real. Broader data access and connectivity must be paired with a demand for strong cybersecurity.

Finally, some basic operational transformation is needed to improve the efficiency of contracting and budget preparation. Facilitating multiple research sites and product sponsors to agree on essential contracting elements and budget parameters, and to efficiently complete the specific negotiations for each study must become a priority. Patients are waiting while paperwork piles up. (See Oncology Clinical Trials Recommendation #3)

Why Agency Action Alone Will Not Be Enough

Operation Trialblazer may be executed faithfully and yet the US may still fall short; some constraints lie outside the reach of FDA or HHS. The agency can clarify its expectations, streamline its reviews, and modernize its guidance documents — and it should. But it cannot appropriate funds to assess and build Phase 1 capacity. It cannot stand up the physical and human infrastructure on which every reform ultimately depends. Some reforms require change at other HHS operating divisions. Those are the province of Congress and, in places, of CMS and other partners.

That is not a counsel of discouragement. It is the opposite. It means that the reforms with the highest leverage are precisely the ones this Subcommittee is positioned to advance. The user fee

reauthorization now on the horizon is a natural, timely vehicle for several of them. The FDA has done, and is doing, its part.

Conclusion

Let me close with the two points. First, HHS has charted a sound course in Operation TrialBlazer, and the stakeholders who will operate within these reforms have reached, independently, many of the same conclusions. Second, the work that remains presents an opportunity for Congress: assessing and strengthening our national Phase 1 capability, removing the statutory and coverage barriers that keep patients out of trials, and resourcing the infrastructure on which the FDA's reforms. The surest way to maintain America's leadership in biomedical innovation is to build — deliberately, and with the tools that reside in this chamber — the strongest system for developing therapies anywhere in the world.

The Reagan-Udall Foundation will continue to do what Congress created us to do: convene the ecosystem, surface the shared challenges, and translate complex questions into terms that patients, developers, and policymakers can all engage. We stand ready to support this Subcommittee and the FDA in that work. Thank you, and I welcome your questions.

Links to Reports cited in this written statement:

1. Improving Oncology Multi-Regional Clinical Trials (https://reaganudall.org/publications/improving-oncology-multi-regional-clinical-trials)
2. Recommendations for Expanding Regulatory Agility and Evidentiary Integrity in Developing Treatments for Rare Diseases (https://reaganudall.org/publications/recommendations-expanding-regulatory-agility-and-evidentiary-integrity-developing)
3. Enhancing Early-Stage Drug Development in the United States (https://reaganudall.org/publications/enhancing-early-stage-drug-development-united-states)