EARLY-STAGE DRUG DEVELOPMENT POLICY RECOMMENDATIONS

For U.S.-Based Clinical Trials

Early-stage clinical trials can be a bottleneck in drug development leading to uncertainty and inefficiency. ACRO is actively working to identify regulatory, structural, and policy barriers and has developed a set of 5 actionable policy recommendations intended to strengthen U.S. leadership in early-stage drug development research.

Regulatory Change | Sub-Regulatory Change | Legislative Change | Behavioral Change | Industry Change

Strengthening FDA Engagement and Clarifying Regulatory and Safety Oversight

How can we make U.S. early-stage clinical trials faster and more efficient without compromising safety? One area with significant potential is strengthening FDA engagement and clarifying centralized oversight.

Several opportunities stand out:

- Publish the Final Rule (proposed in 2022) requiring any U.S. institution participating in FDA-regulated cooperative (multi-site) research to rely on approval by a single IRB for that portion of the research that is conducted in the U.S.
- Reinforcement and education from FDA around requirements and guidance for clarity and implementation.
- Utilize agency convening power to encourage cross-stakeholder efforts such as standardized contracting, common operational metrics, and accelerated site activation targets.

Impact:

While these changes may not fully eliminate the startup gap between the U.S. and other regions, they could meaningfully reduce operational friction, improve predictability, and accelerate the path from scientific discovery to patient access.

Move Toward a Risk-Tiered, Adaptive Regulatory Framework for Early Stage Trials

As global competition for early-phase research intensifies, the U.S. must consider how oversight can become more efficient, predictable, and proportionate to risk. This policy priority focuses on a risk-tiered, adaptive regulatory framework for early-stage trials that can help accelerate development while maintaining strong safety standards.

Potential areas for exploration include:

- Deploy a risk-tiered IND pathway with clear, objective criteria and review timelines.
- Expand regular opportunities for applicant communication with FDA, ensuring faster and more predictable regulatory interactions.
- Increase FDA resources to strengthen scientific advice capacity and expertise for applications containing novel compounds and increasingly complex research methods and designs.
- Greater flexibility for adaptive trial designs, dose-escalation strategies, and rolling reviews.
- Streamlined approaches to safety reporting and protocol amendments.
- Broader adoption of adaptive and model-informed drug development approaches.

Impact:

A more adaptive approach aligned with these areas could help strengthen the U.S. as a destination for early-stage clinical research.

Modernize Expectations Around AI-Enabled Trial Execution

FDA is already a global leader in setting expectations for the computational methods reshaping early development, from pharmacometric modeling and simulation to AI-derived and model-informed approaches. The opportunity for HHS is to resource and sustain that lead so these methods make early-stage development faster, more efficient, and less burdensome on volunteers. This policy priority focuses on ensuring clear implementation guidance around AI to reduce uncertainty.

Where support would matter most:

- Fund FDA's quantitative and computational review and inspection capacity.
- Sustain and broaden structured scientific engagement on model-based approaches.
- Finalize predictable validation expectations.

Impact:

Ensuring a stable, transparent regulatory environment converts a real U.S. scientific strength into faster, more efficient early development, with more of this science conducted and reviewed here while maintaining high standards for quality, safety, and data integrity.

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Address Structural and Economic Realities Impacting U.S. Competitiveness

We know that decisions about where to conduct early-stage studies are driven by cost, infrastructure, and speed as much as by regulation. If we want more early-stage trials in the U.S., regulatory reform alone won't be enough. This policy priority focuses on addressing the structural and economic factors that impact U.S. competitiveness.

Potential solutions could include:

- Meaningful, timely financial incentives to attract early-stage research, such as refundable R&D tax credits.
- Ensure a more predictable early-stage regulatory environment

The goal should be to make the U.S. a more attractive location for conducting early-stage clinical research without imposing undue burden.

Impact:

Targeted economic and infrastructure investments in all parts of the ecosystem may have as much impact on U.S. competitiveness as regulatory process improvements.

Reduce Participant Burden and Recruitment Barriers

As the industry looks for ways to improve participation in clinical trials broadly, it's worth examining policies that create unnecessary barriers for volunteers. This policy priority focuses on reducing participant burden.

Potential opportunities include:

- Clarity from FDA recognizing risk-based protocol flexibility, taking into account procedure intensity and patient burden.
- Flexible enforcement of eligibility criteria to mitigate unnecessary restrictions and ensure focus on enforcement of requirements identified by the safety/risk profile.
- Public awareness initiative aimed at increasing patient and provider awareness of and guidance on clinical trial opportunities.
- Simplify and modernize the participant consent process.
- Require insurance coverage for routine clinical trial costs.
- Exempt participant stipends from taxable income.

Impact:

Creating a more participant-centered clinical trial ecosystem could improve recruitment, reduce barriers to participation, and help accelerate the development of new therapies.

ABOUT ACRO

Founded in 2001, the Association of Clinical Research Organizations (ACRO) is non-profit trade association representing the world's leading clinical research and technology organizations, which provide specialized services that are integral to the development of drugs, biologics and medical devices that enable patients to live longer, healthier, and more productive lives. ACRO expertise covers the entire spectrum of development – from preclinical, proof of concept, and first in human studies through post-approval, pharmacovigilance, and health data research. ACRO member companies employ nearly 470,000 people worldwide and conduct research in more than 90 countries in every global region.