Phase 1 Trials: Policy and Action

The Duke Clinical Research Institute (DCRI) Think Tank is an interactive meeting that engages diverse perspectives and leadership expertise to address key issues in clinical research, policy, and practice to improve health.

On January 23, 2026, the DCRI convened a group of experts to examine the increasing shift of early clinical development, including Phase 1 and first-in-human studies, away from the United States (U.S.) and notably towards China, where a state-directed biomedical strategy is driving rapid growth in early-phase research. Attendees represented a broad range of organizations, including academic institutions, federal agencies, congressional offices, clinical research organizations, policy and non-profit organizations, biotechnology companies, and investment organizations. Dialogue centered around key factors driving the shift, the long-term implications for U.S. biomedical innovation and competitiveness, and policy and operational strategies to strengthen U.S. early-phase clinical development. Discussions examined differences in regulatory, ethical and operational strategies, bottlenecks in U.S. Phase 1 operational processes, international models for accelerating early-phase research, and approaches to maintain patient safety while improving U.S. speed, cost efficiency, and competitiveness in executing early-phase clinical trials.

KEY TAKEAWAYS AND THEMES:

- **Learning from other sectors: China utilizes supply chain dominance for strategic leverage, and the biopharmaceutical industry is now a target.** China has utilized a state-directed policy to achieve market dominance in industries such as rare earth minerals and electric vehicles, potentially controlling access to critical materials to achieve political and industrial objectives. Through initiatives such as Made in China 2025, now extended through the decade, China aims to localize production of key industrial and technological products. This approach is now being applied to the pharmaceutical sector, first in manufacturing and increasingly in research. By executing more efficient and cost-effective Phase 1 clinical development, China incentivizes U.S. companies to conduct early-stage trials in their country, thereby giving Chinese firms insights into innovations and the ability to replicate and improve them. This creates an asymmetrical dependency that weakens early clinical development capabilities in the U.S. and increases reliance on China for early-phase clinical research. Without proper U.S. incentives, guardrails and regulation, China's growing capabilities could eventually restrict U.S. access to critical medicines.

- **Ensuring data quality, transparency, ethical and scientific integrity in clinical trials conducted outside the U.S. remains a significant challenge.** Structural differences of clinical practice outside the U.S. including government-driven healthcare, unclear patient autonomy in medical decisions, and varying standards for voluntary informed consent create differences in patient care that may result in reduced ethical standards and limited oversight of trial conduct. These factors can have implications for data quality, generalizability, and adherence to Good Clinical Practice (GCP) standards.

- **U.S. biotech and pharmaceutical companies conduct early-phase trials outside the U.S. to accelerate development, reduce costs, and generate human data while navigating slower, more resource-intensive processes in the U.S.** One key contributor is the U.S. approach to extensive pre-IND requirements such as Chemistry, Manufacturing, and Controls (CMC), under which Phase 1 trials are subject to intensive up-front requirements despite substantial uncertainty about clinical benefit. When combined with early Investigational New Drug (IND) feedback that extends beyond assessing whether a trial is safe to proceed, these practices collectively result in up to years of delay in trial initiation in comparison to other countries. Australia's Clinical Trial Notification (CTN) system provides rapid institutional ethics review and regulatory notification, enabling trials to begin less than 70 days after final protocol. Australia also offers moderate per-patient Phase I costs, a substantial tax incentive, high-quality data, simplified contracting, high-performing sites, and predictable activation timelines along with a remarkable safety record. During this pre-IND approval time, China and Australia would already have enerated human safety, dosing, and early efficacy data.

- **Domestic reform efforts have begun to accelerate U.S. biotech research and early-phase clinical trials, but there remains significant room to improve efficiency and oversight.** Key initiatives include speeding Phase 1 trials by clarifying evidence standards and improving platform designations for manufacturing to reduce CMC efforts and other requirements in the pre-IND application. However, there is still room for improvement and systems such as Australia's Clinical Trial Notification System with fit-for-purpose regulatory submissions and streamlined ethical review present viable learning opportunities for the U.S. system.

ACTIONABLE ITEMS

Establish a U.S. National Strategy for Early-Phase Trials:

- Make maintaining global leadership in early-phase clinical research a national priority by defining clear goals for U.S. leadership in Phase 1 clinical research and aligning regulatory, industry, and funding priorities.
- Frame the problem around patient access to new cures, innovation speed, and global competitiveness, particularly relative to China. Australia's CTN pathway is a good example of a national strategy that can be improved on to re-establish U.S. as a leader in early clinical development.
- Explore opportunities to incentivize U.S. investment in early-phase research, particularly in strategically important areas (e.g. chronic health conditions; cancer), through financial mechanisms such as tax credits, rebates, or government-backed purchasing.

Streamline Pre-IND and Phase 1 Processes:

- Identify which elements of systems like Australia's Clinical Trial Notification framework (e.g., ethics committee review, fit-for-purpose regulatory submission requirements) can be adapted for the U.S. and establish clear guidance on how these components should be applied to accelerate first-in-human trials while ensuring patient safety and adherence to GCP standards.
- Establish phase-adapted IND requirements, such as fit-for-purpose CMC standards, to reduce the regulatory burden on early-phase studies.
- Establish guidelines for Institutional Review Board (IRB) review to streamline ethical review and reduce redundancy.
- Centralize U.S. site budgeting, contracting, and administrative processes by implementing standardized templates for burdensome processes such as budgeting and contracts.
- Establish public reporting of common clinical trial metrics such as timelines to start-up trials to improve the eco-system.
- Create a safe harbor for early phase research at U.S. sites.
- Consider broader policy levers, including Intellectual Property (IP) reform opportunities.
- Invest in the translational science eco-system in the U.S. to be more automated to accelerate pre-clinical studies.

Enhance Data Transparency and Regulatory Confidence:

- Implement authorized inspections or pre-certification processes for sites in countries of concern to verify adherence to GCP standards. Explore verification strategies, such as remote monitoring, to maintain compliance at sites with limited on-site access.
- Strengthen data quality verification of foreign early-phase data through data requirements such as patient-level data and rigorous consent documentation.
- Leverage FDA IND enforcement proactively by clearly communicating the risk of IND withdrawal for noncompliant studies to ensure adherence to GCP standards and a signal of regulatory rigor to sponsors conducting trials in countries of concern.

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