Next-Generation U.S. Clinical Development to Accelerate Cures - DRAFT

Legislative Summary

Overview

This legislative proposal aims to modernize the clinical development of innovative biomedical products to match the accelerating pace of discovery and preclinical development of breakthrough innovations, through **implementing clinical trial platforms** to enable the shift from a **separate, fragmented system** into a **core function of U.S. health** care, and through linking more efficient clinical trial capabilities to **modernized Phase 1 clinical development** and opportunities to advance these reforms through **global regulatory alignment**.

This proposal addresses these aims through the **Cures in Care Initiative (CCI)** to provide a pathway to embed clinical trials into routine U.S. health care delivery and a complementary set of proposals for **Modernized Preclinical and Early-Stage Clinical Development** and **Enhanced FDA Transparency.** These reforms together can modernize the clinical development process, turning promising scientific breakthroughs into faster and broader access to cures for unmet health needs in the U.S. and worldwide.

This proposal accelerates a **structural modernization of how clinical trials are conducted and how early-stage clinical development occurs.** It builds on existing federal authorities and proven models to scale and emphasizes:

- **Use of existing authorities** with targeted practical steps to facilitate lead point-of-care and clinical trial “platform” approaches to integrating clinical research into routine care
- **Public-private and cross-agency collaboration** to improve clinical research and clinical care
- **Pilot-based scaling with measurable outcomes** in lowering trial costs, accelerating enrollment, expanding patient access, and enabling U.S. leadership in global clinical development reforms

In the **near term**, the proposed legislation will:

- Launch regional and national pilot trial platforms
- Identify and implement targeted regulatory updates and leverage emerging CMS/ONC data and interoperability standards to facilitate real-world clinical research
- Implement key modernizations in Phase 1 testing to reduce costs and accelerate clinical launches

In the **medium term** (by 5 years), the proposed legislation will:

- Scale lead platforms and extend platform capabilities to support research embedded in clinical care to a broader range of high-burden disease areas and health care organizations
- Integrate routine use real-world data and studies into product approvals, labeling, and other relevant evidence to accelerate the impact of transformative therapies
- Advance the adoption of aligned reforms in other regulatory agencies, to enable a more efficient, reliable, and confident global regulation of innovative medical products

Purpose

Modernize U.S. clinical development by embedding clinical trials into routine care and modernizing Phase 1 clinical development, enabling faster, lower-cost, and more accessible clinical development of promising new treatments for unmet needs.

Key Provisions

1. Short Title

Cures in Care Initiative (CCI): Embedding Clinical Studies into Routine Health Care

2. Cross-Agency Support for the Cures in Care Initiative (CCI) to Embed Clinical Trials in Routine Care ("Point of Care" Study Participation)

- Assigns the CDER Center for Clinical Trial Innovation as the centralized coordinating entity within HHS to establish reusable, point-of-care clinical trial platform networks
- Authorizes a new pathway to scale point-of-care trial networks to accelerate promising treatments and innovative methods for diseases with significant unmet medical need
- Sets a timeline of 1 year to determine initial networks for participation and 3 years for broader-based adoption, with further goal setting to realize expanded access, reduced costs and impact
- Improves upon existing FDA regulations, guidance documents, and authorities to sustainably scale reusable clinical trial platforms by modernizing IRB oversight, ethical conduct of research, innovative methods, dissemination of findings, governance and use of new technologies
- Facilitates private and public stakeholder cooperation
- Identifies best practices for training and technical support for health care organizations—focusing on institutions with less capacity and capability—to join reusable clinical trial platforms

3. Agency Actions to Create and Expand Point-of-Care Infrastructure

- Aligns regulatory requirements for real-world clinical research data with emerging CMS/ONC data and interoperability standards
- Accelerates initiatives to validate biomarkers and clinical outcomes assessments relevant to reducing cost and time of point-of-care platforms, and align with use of such measures in federal health care quality improvement initiatives

- Requires an interagency implementation plan and an FDA guidance revision addressing cross-agency data modernization and the use of digital/AI tools in point-of-care trials

4. Implementation and Scaling of Point-of-Care Platforms

- Directs ARPA-H/NIH implementation of at least 3 lead point-of-care platform opportunities, including applications in rare diseases, high-burden chronic diseases, and other significant unmet needs
- Establishes criteria emphasizing feasibility for implementation by non-academic center health care providers and providers serving high-risk patient populations, and participation by a critical mass of biopharma product developers
- Authorizes appropriations for CCI activities and platform awards to support the lead pilots and federal technical support for enabling regulatory reforms [including NIH, ARPA-H, and potential user fee sources]
- Demonstrates support for a CCI Collaborative for rapid learning and evaluation to increase the initiative's impact

Modernized Preclinical and Early-Stage Clinical Development

5. Incorporation of New Approach Methodologies (NAMs) in Preclinical and Early Clinical Development

- Instructs FDA to issue guidance and regulatory frameworks to further adoption of in vitro and in silico technologies to enhance preclinical and early clinical research through determining lead use cases, detailing data needed for validation, identifying pathways for validation to support rare disease therapeutics and advancing efforts to reduce animal testing
- Increases the use and reliability of NAMs through pre-competitive cooperation among trial sponsors and preclinical research organizations to incentivize knowledge sharing of validation methodologies
- Leverages the lessons from this pre-competitive collaboration to support FDA-led actions—in coordination with NIH and other relevant HHS components, ARPA-H—to expedite validation of promising NAMs

6. Modernized Phase 1/First-in-Human Clinical Trial Oversight

- Directs FDA to establish pilot programs for third-party oversight of lower-risk Phase 1 trials—modeled after Australia's CTN scheme
- Establishes guidance for pre-certification of third-party organizations implement this IND alternative pathway
- Develops additional guidance and pilots for best practices to ensure rigorous oversight by IRB or other organizations
- Requires HHS to assess whether Phase 1 pilot outcomes warrant Common Rule revisions
- Adds considerations for high-risk phase 1 trials including proportionate requirements for study conduct, monitoring, and manufacturing; guidance and pilots for central IRBs; and dissemination of findings to explore a pre-certification process for high-risk studies

Aligned Global Modernization of Clinical Development

7. Global Regulatory Alignment

- Develops and advances initiatives for FDA to lead on global regulatory alignment to modernize best practices for clinical development
- Leverages existing forums to progress priority steps to modernize early-stage clinical development and reusable, point-of-care clinical trial platforms
- Improves oversight of foreign trials through authorized inspection authorities, enhanced data quality verification, and issuing of guidance on limitations for sites that inhibit inspections

8. Adequate U.S. Representation in Clinical Trials

- Establishes a requirement for clinical data to be collected in the US, with exceptions reviewed by the HHS Secretary for GCP compliance and applicability of the data to the US health care system
- Calls for patients in the US to be enrolled in a multinational platform trial

Enhanced FDA Transparency to Support Modernization

9. Rare Disease Innovation and Public Engagement

- Codifies the FDA Rare Disease Innovation Hub (RDIH), with specified duties and funding mechanisms to ensure further guidance, support for rare disease therapeutic studies, and efforts to develop individualized therapies
- Directs the RDIH to coordinate engagement and consistency across FDA Centers and rare disease programs, partake in CCI, and collaborate with other pilots and programs that support rare disease therapeutic development
- Authorizes a clinical outcome assessment development and validation program focused on more efficient clinical studies

10. Resolving Clinical Holds and Improving Trial Efficiency

- Advances early engagement by the HHS Secretary to determine frequent areas of noncompliance and apply best practices for GCP compliance through formal meetings between trial sponsors and FDA
- Authorizes additional resources through appropriations
- Requires the GAO to issue a report on the use of existing FDA drug development resources to advance clinical trial efficiency and consistency of regulatory guidance for rare disease therapeutic development

11. Mutual Recognition of GMP Standards

- Enhances adoption of shared inspection reports by FDA from jurisdictions that effectively enforce GMP standards
- Develops strategies for prioritizing the frequency and a risk-based for inspections

12. Evidence-Based Regulatory Reforms for Follow-On Product Sponsors

- Directs FDA to improve the Competitive Generics Pathway through engagement of health care stakeholders
- Strikes the interchangeability legal designation for biosimilar products
- Establishes a 505b(2) pathway for follow-on biologics

For more information or to share a comment, please feel free to reach out to Nikita Varman, Health Policy Advisor, Congressman Auchincloss (MA-04) at nikita.varman@mail.house.gov.
