Artificial Intelligence in Drug Development: Use Cases, Challenges, and Future Considerations

CLINICAL TRIALS TRANSFORMATION INITIATIVE

MEETING SUMMARY

MEETING OBJECTIVES

The second annual public workshop on artificial intelligence (AI) in drug and biological product development – hosted by the FDA in collaboration with CTTI – convened experts across the clinical research enterprise to discuss the rapidly evolving role of AI in advancing the safety, efficacy, and quality of drug and biological product development. Discussions focused on current and emerging AI use cases across the product lifecycle, the data infrastructure needed to support fit-for-purpose AI models that are trustworthy and representative, and the regulatory considerations for AI used to support decision-making in clinical trials. Attendees explored barriers to the integration of AI in clinical development pipelines and potential collaborative strategies for accelerating innovation while protecting patient safety and privacy.

MEETING THEMES

Throughout the meeting, attendees discussed use cases illustrating how AI is transforming drug and biological product development, reflected on persistent barriers making integration of AI into clinical development challenging, and considered strategies for supporting reliable, trustworthy, and fit-for-purpose AI-driven drug development. The following key themes emerged:

- **AI is Transforming Drug Development:** AI can fundamentally change how new therapies are discovered, clinical trials are designed, and patient outcomes are measured. It can enable data-driven decision-making, optimize trial protocols, and support the development of digital biomarkers and patient-centered endpoints.
- **Collaboration and Data Sharing Are Essential:** Progress in AI-driven drug development requires robust collaboration across public and private sectors, secure data-sharing frameworks, and transparent mechanisms for exchanging lessons learned. Federated learning and shared responsibility frameworks can help overcome data silos and intellectual property barriers.
- **Regulatory Reform and Risk-Based Approaches:** The FDA has established a risk-based framework for evaluating AI in drug and biological product development. Early engagement with regulators, transparency, and comprehensive documentation tailored to the risk profile of each AI model are critical for responsible innovation.
- **Data Quality, Reliability, and Representativeness:** Reliable data pipelines, foundation models, and robust infrastructure are foundational for trustworthy AI. Ensuring data quality, representativeness, and access – while protecting patient privacy – is essential for building fit-for-purpose AI that reflects real-world representativeness and avoids bias.

NEXT STEPS

Meeting attendees reflected on potential strategies for advancing the integration of AI in drug and biological product development:

- **Regulatory Clarity and Collaboration:** Clear, harmonized regulatory frameworks and early engagement with agencies like the FDA are critical for responsible AI adoption. Ongoing collaboration among regulators, industry, academia, and patient advocacy groups is needed to establish best practices, build trust, and address evolving challenges in AI credibility and documentation.

- **Data Quality and Infrastructure:** Investment in computational resources, data infrastructure, and integration of data scientists into development teams will drive progress, while systematic data generation and validation help ensure model reliability and meaningful patient outcomes.
- **Trust, Transparency, and Human Oversight:** Building trust in AI systems requires explainability, interpretability, and user-centric design. Human oversight, continuous validation, and transparent documentation are necessary to ensure AI models remain accurate, relevant, and safe throughout their lifecycle.
- **Ethical, Patient-Centered, and Multidisciplinary Approaches:** Responsible AI use in drug development demands strong ethical principles, ongoing collaborative partner engagement (including patients), and multidisciplinary collaboration. Addressing health disparities, ensuring data privacy, and fostering a culture of transparency and innovation are ongoing priorities.

Meeting materials, including agenda, recording, and slides are included on CTTI's Artificial Intelligence in Drug & Biological Product Development Workshop webpage.

ABOUT THE CLINICAL TRIALS TRANSFORMATION INITIATIVE (CTTI)

The Clinical Trials Transformation Initiative (CTTI), a public-private partnership co-founded by Duke University and the FDA, seeks to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials. Bringing together organizations and individuals from across the enterprise, CTTI is transforming the clinical trials landscape by developing evidence-based solutions to clinical research challenges.

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