Regulatory Perspectives on the FDA's Use of Artificial Intelligence in Drug Development

As artificial intelligence (AI) gains prominence in drug development, regulatory agencies such as the US Food and Drug Administration (FDA) are creating and implementing frameworks to promote the responsible use of AI for medical products. The FDA defines AI as a machine-based system that can, for a given set of human-defined objectives, make predictions, recommendations, or decisions influencing real or virtual environments.[1]

A perspective article published in *JAMA* in January 2025 noted that the FDA's first approval of a partially AI-enabled medical device occurred in 1995 for PAPNET, a software used to prevent misdiagnosis in women undergoing Papanicolaou tests for cervical cancer.[2] The article indicated that, since the approval of PAPNET, the agency has authorised approximately 1,000 AI-enabled medical devices. The authors, including former FDA commissioner, Robert M. Califf, MD, stated that to "keep up with the pace of change," in AI across biomedicine and healthcare, regulators will have to "advance flexible mechanisms." While the agency has developed a total life cycle approach to support the deployment and innovation of AI-enabled products, industry and other external stakeholders will need to "ramp up," their evaluation and quality management of AI "beyond the remit of the FDA."

Efforts to Establish Policies for AI

At the 2025 annual Drug Information Association (DIA) meeting, Tala Fakhouri, PhD, MPH, Associate Director for Data Science and AI at the FDA's Center for Drug Evaluation and Research (CDER), presented some of the agency's efforts to establish policies on AI. She highlighted that CDER has received 800 product submissions with AI components from 2016 to 2024. Since 2019, CDER started documenting the use of AI in regulatory submissions, created a working group, issued discussion papers, and convened several workshops, including those in collaboration with the Duke-Margolis Institute for Health Policy in 2022 and the Clinical Trials Transformation Initiative (CTTI) in 2024.[3,4]

One of the discussion papers described the four main areas of focus regarding the development and use of AI for several of the FDA's organisations, including CDER, the Center for Biologics Evaluation and Research (CBER), the Center for Devices and Radiological Health (CDRH), and the Office of Combination Products (OCP).[5] These areas are to 1) foster collaboration to safeguard public health; 2) advance the development of regulatory approaches that support innovation; 3) promote the creation of harmonised standards, guidelines, best practices, and tools; and 4) support research related to the assessment and monitoring of AI performance.

As another relevant resource, in January 2025, the FDA also published the draft guidance for industry, *Considerations for the Use of Artificial Intelligence to Support Regulatory Decision-Making for Drug and Biological Products,* which provides recommendations to interested parties on the use of AI to produce information or data to support regulatory decision-making regarding safety, effectiveness, or quality for drugs.[6] The guidance outlines a risk-based credibility assessment framework comprising a 7-step process, and it encourages sponsors to "engage early," with the FDA to set expectations about the framework and help identify potential challenges.

Looking ahead, Fakhouri explained that the FDA will continue to refine its January 2025 AI guidance, which had received 1,450 comments from 98 entities by the time of her presentation. Moreover, the agency will focus on addressing stakeholder requests for issuing guidance on good machine learning practices, harmonising terminology, and engaging with all interested parties, including through a future CTTI workshop in 2025.

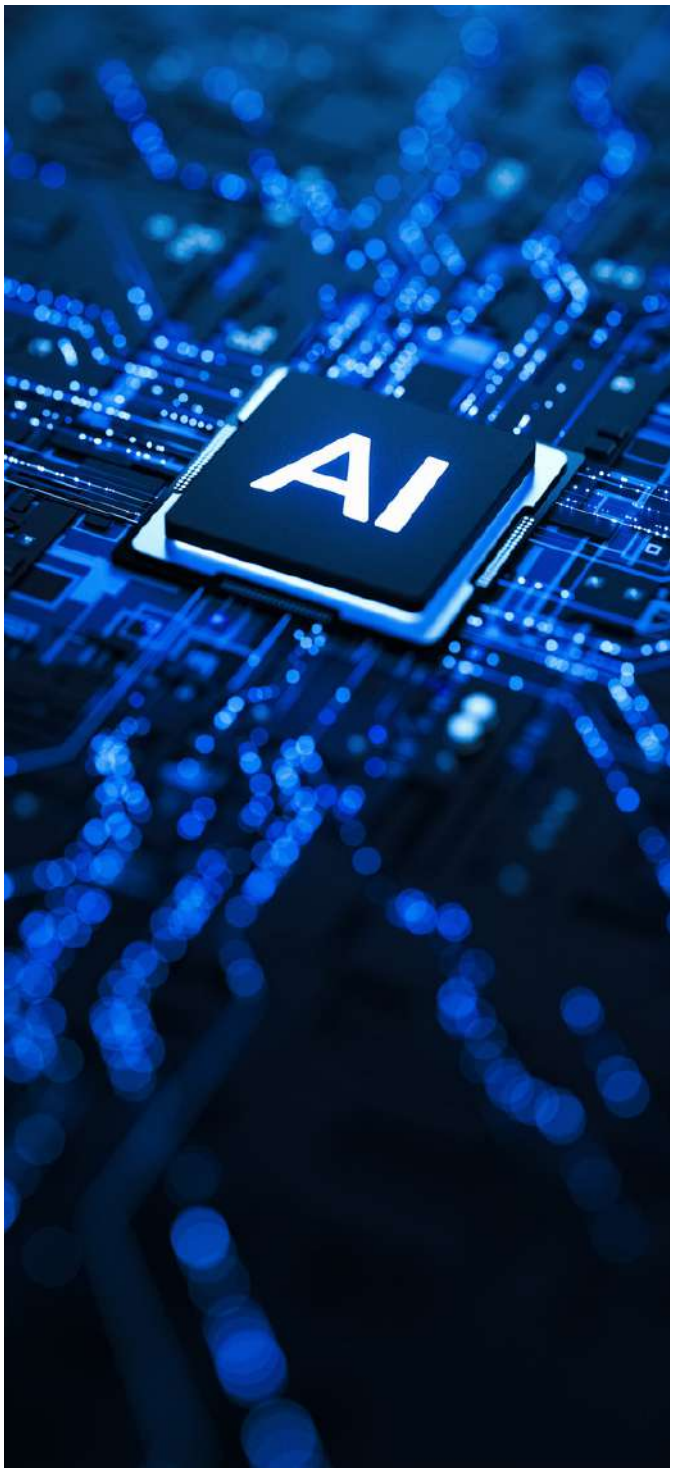
Integrating Generative AI for Internal Use

Within the FDA, there has also been a focus on the use of AI internally. On May 8, 2025, FDA Commissioner Martin A. Makary, MD, MPH, announced the completion of an AI-scientific review pilot and shared an "aggressive timeline," for the agency to deploy a generative AI tool across all FDA centers by June 30, 2025.[7] Named Elsa, the tool was launched on June 2, 2025.[8] The FDA press release about Elsa explained that it was built within a high-security GovCloud environment and offers a secure platform for FDA staff to access internal documents while housing the information within the agency. Some of the tasks Elsa can perform include summarising adverse events, conducting faster label comparisons, and generating code to develop databases for nonclinical applications, the agency highlighted.

At DIA 2025, Fakhouri provided information on Elsa. She clarified that regulatory submissions would continue being evaluated by humans and that FDA reviewers are generally using Elsa to perform administrative tasks to increase their efficiency. Jeremy Walsh, the FDA's newly appointed chief AI officer, also offered his perspective, explaining that Elsa is not being trained on data submitted to the agency. This approach ensures that only FDA staff handle this sensitive data. However, he stated that some external stakeholders have expressed interest in training Elsa with this data and that this topic would likely be explored in the future. The FDA panelists at the conference mentioned that the agency is interested in sharing findings and prompts with stakeholders and encouraged collaboration and transparency on the development of AI tools.

REFERENCES

1. Artificial Intelligence and Medical Products. Food and Drug Administration webpage. https://www.fda.gov/science-research/science-and-research-special-topics/artificial-intelligence-and-medical-products
2. Warraich HJ, Tazbaz T, Califf RM. FDA Perspective on the Regulation of Artificial Intelligence in Health Care and Biomedicine. JAMA. 2025; 333(3):241-247. https://pubmed.ncbi.nlm.nih.gov/39405330/
3. Understanding AI/ML in the Drug Development Lifecycle. Duke-Margolis Institute for Health Policy. https://healthpolicy.duke.edu/events/understanding-aiml-drug-development-lifecycle
4. Artificial Intelligence (AI) in Drug & Biological Product Development Hybrid Public Workshop. Clinical Trials Transformation Initiative. https://ctti-clinicaltrials.org/type/news/fda-ctti-convening-hybrid-public-workshop-on-artificial-intelligence-in-drug-biological-product-development/
5. Artificial Intelligence & Medical Products: How CBER, CDER, CDRH, and OCP are Working Together. Food and Drug Administration. https://www.fda.gov/media/177030/download?attachment
6. Considerations for the Use of Artificial Intelligence to Support Regulatory Decision-Making for Drug and Biological Products. Food and Drug Administration webpage. https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-use-artificial-intelligence-support-regulatory-decision-making-drug-and-biological
7. FDA Announces Completion of First AI-Assisted Scientific Review Pilot and Aggressive Agency-Wide AI Rollout Timeline. Food and Drug Administration webpage. https://www.fda.gov/news-events/press-announcements/fda-announces-completion-first-ai-assisted-scientific-review-pilot-and-aggressive-agency-wide-ai
8. FDA Launches Agency-Wide AI Tool to Optimize Performance for the American People. Food and Drug Administration webpage. https://www.fda.gov/news-events/press-announcements/fda-launches-agency-wide-ai-tool-optimize-performance-american-people



Jennifer Nguyen

Jennifer Nguyen, PhD, is a Senior Content Analyst for the Cortellis suite of life science intelligence solutions at Clarivate. She previously worked as a medical writer, which involved writing and editing scientific journal articles and materials for science conferences. Her current role includes reporting on FDA advisory committee meetings, regulatory policies, and workshops.

Email: jennifer.nguyen@clarivate.com